Report to Society

2024

pharma.be

ASSOCIATION GÉNÉRALE DE L'INDUSTRIE DU MÉDICAMENT
ALGEMENE VERENIGING VAN DE GENEESMIDDELENINDUSTRIE

Foreword

2024 was an eventful year, in which new governance teams had to be established at the European, federal, and regional levels. All face significant challenges. Competitiveness, economic growth, and employment need strengthening, while people and nature require care. At the same time, public finances need to be put in order.

In the European Competitiveness Report by Mr. Draghi, published in September 2024, the pharmaceutical industry was identified as strategically important. Not only does our sector contribute to economic prosperity, but it also acts as a powerful driver of public and patient health, which becomes increasingly vital for an ageing population. The revision of European pharmaceutical regulations this past year aimed, among other things, to strengthen Europe's position. However, there are still many areas in EU policy that could be improved, as highlighted by the Draghi report Innovation will continue, but the question remains: where? If Europe's position deteriorates, that would be bad news for Belgium, as our footprint within the European framework is considerable.

This is further evidenced by the achievements presented by pharma.be in this fourth "Report to Society". Over the past year, Belgium's pharmaceutical industry retained a leading position within the European Union in research and development, production, and export. Yet Europe's own position in the ranking of innovative regions is declining. For instance, if we look at the number of clinical studies conducted on cell, tissue, or gene therapies, it's evident that countries like the US and China have moved far ahead of us compared to 10 years ago.

Belgium ranks number one in Europe for research and development (R&D) investment per capita, with the sector investing €15.5 million daily in R&D. We rank number two in terms of clinical studies per capita, patent applications per capita, and value added per employee. Furthermore, we are third in Europe for job creation.

Caroline Ven
CEO pharma.be

If our country wants to foster innovation, it must continue to take measures that encourage scientific advancement. Without incentives for innovation, many of the medicines and vaccines we have today simply would not exist. These new medicines are crucial for patients, but they must also be made widely and quickly available. Compared to neighbouring countries, Belgium is slow in reimbursing new treatments. This needs to be improved, and it can be. Together with patient associations and other partners in the healthcare ecosystem, pharma.be has tirelessly advocated for this over the past year and will continue to do so.

In 2023 (the latest data available), 98 new medicines were reimbursed. About half are new medicines for cancer or immunomodulating agents. In addition, new medicines were also made available for nervous and respiratory system disorders – innovations that are very encouraging for patients and their families. This is the ultimate aim of the pharmaceutical industry: more and better treatments for improved public health.

Enjoy (re)discovering the many activities in this fascinating sector!

Table of contents

Who we are 1

1.1 Mission of pharma.be

As a **knowledge centre and representative association** of the **innovative biopharmaceutical industry**, pharma.be acts as a **reliable partner** to contribute to the **sustainable health of citizens, patients, and the economy** in Belgium through **knowledge-sharing, collaboration, and dialogue**.

1.2 Focus on the patient

pharma.be is the ambassador of innovative biopharmaceutical companies in Belgium and fosters a favourable business environment in a responsible manner. The aim is to make sure that patients have optimal access to therapeutic innovation. In this way, we also contribute to improving the Belgian healthcare system.

The manifesto of the biopharmaceutical industry in Belgium

Living your life to the fullest. Spending time with family and friends. Enjoying good health. We all have these needs. They define the way we live our lives. They drive us in everything we do, every single day.

We directly employ about 43,000 people at 126 companies focused on the research and development (R&D) of innovative medicines and vaccines.

For us, health is central: we want the best possible life for everyone in Belgium. That is why our mission is to make Belgium the healthiest place to grow up, live, work and age through innovative health solutions.

We are deeply rooted in science. History shows that the most crucial breakthroughs in healthcare happen in laboratories. Breakthroughs that have improved the treatment of breast cancer or turned HIV into a manageable chronic condition.

But science is a means, not an end in itself. Science is our passion, but only because it allows us to make a positive impact on lives. We are the people who care for others.

The world is changing at a rapid pace: with new diseases and viruses, an ageing population and ever-growing digitalisation. And there are numerous new questions. Scientific progress rarely follows a straight line. In our industry, we fail far more often than we succeed. There is nothing we can do about that. But being scientists at heart, we cannot just accept that. We never give up. It is our responsibility to continue to strive for the best health for all.

We are not alone. Together with patients, doctors, hospitals, accredited health insurance funds, pharmacists, universities, research groups, public authorities and the government, we are united by a common goal: the best possible healthcare for all people in Belgium.

Together, we make sure that everyone can live a healthy life.

1.3 With 126 members

pharma.be brings together **126 biopharmaceutical companies** operating across Belgium. They are present in every key aspect of the biopharmaceutical value chain: **from R&D and clinical trials to production, from market introduction to distribution of medicines.**

Group.10: driving innovation and health

Within pharma.be, *Group.10* unites more than 70 small- and medium-sized enterprises, start-ups and biotechnology companies. They are active in research and development, clinical trials, production and distribution of medicines.

Together, they represent:

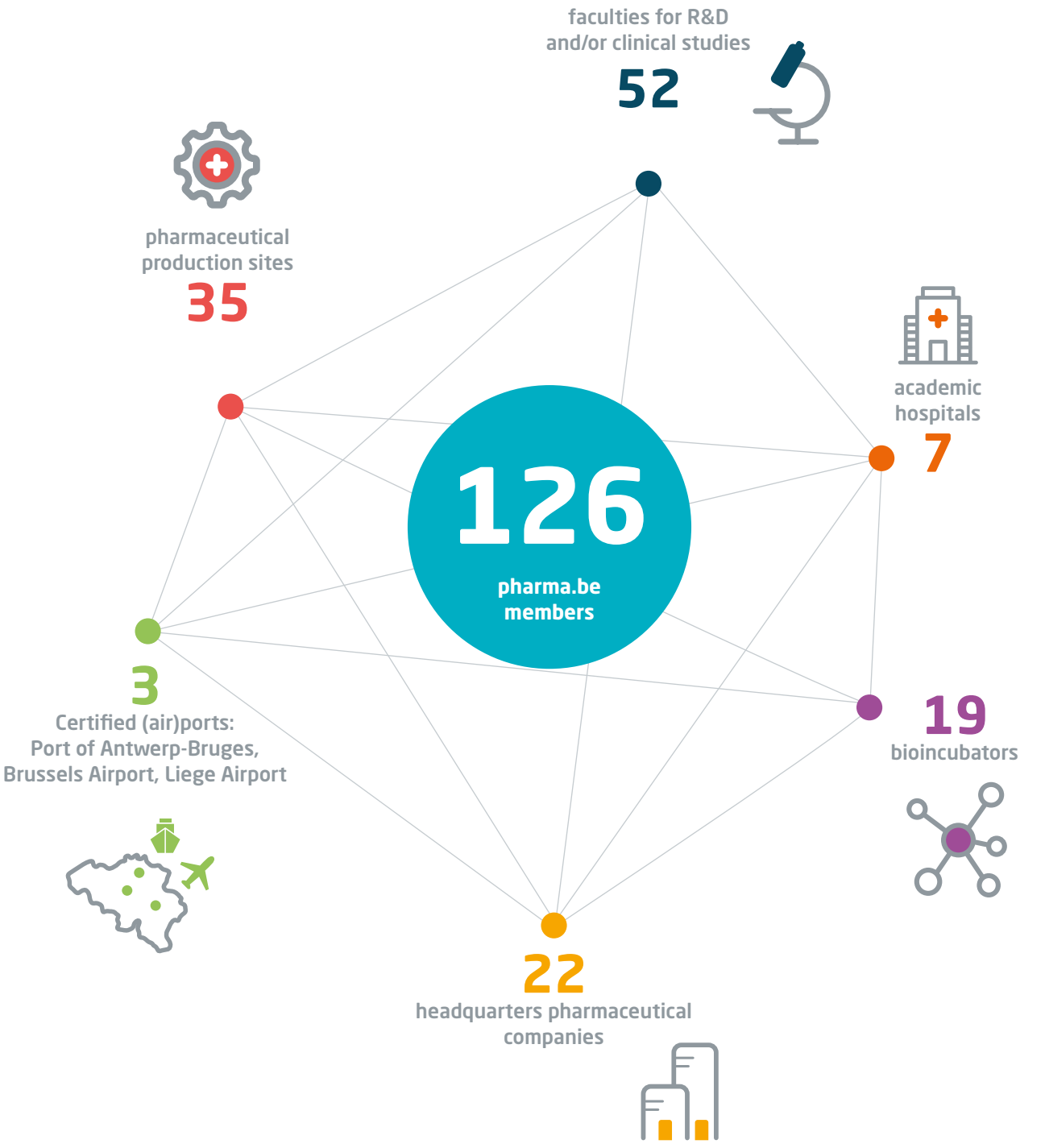
- **± 10%** of the turnover of the innovative biopharmaceutical industry in Belgium
- **3,382 jobs** in 2023
- **629** medicines on the market in 2023
- **€477,119** added value per employee in 2023

Source: pharma.be, based on IQVIA and Bel-first

These smaller biopharmaceutical companies may be more affected by shifts in policy and market conditions, but they are a driving force for innovation and health.

1.4 As part of a large ecosystem

The members of pharma.be are part of a **Belgian biopharmaceutical ecosystem**, together with universities and strategic research institutes, teaching hospitals and pharmaceutical incubators, as well as logistics players including airports and the Port of Antwerp-Bruges.



Source: pharma.be

1.5 Committed directors

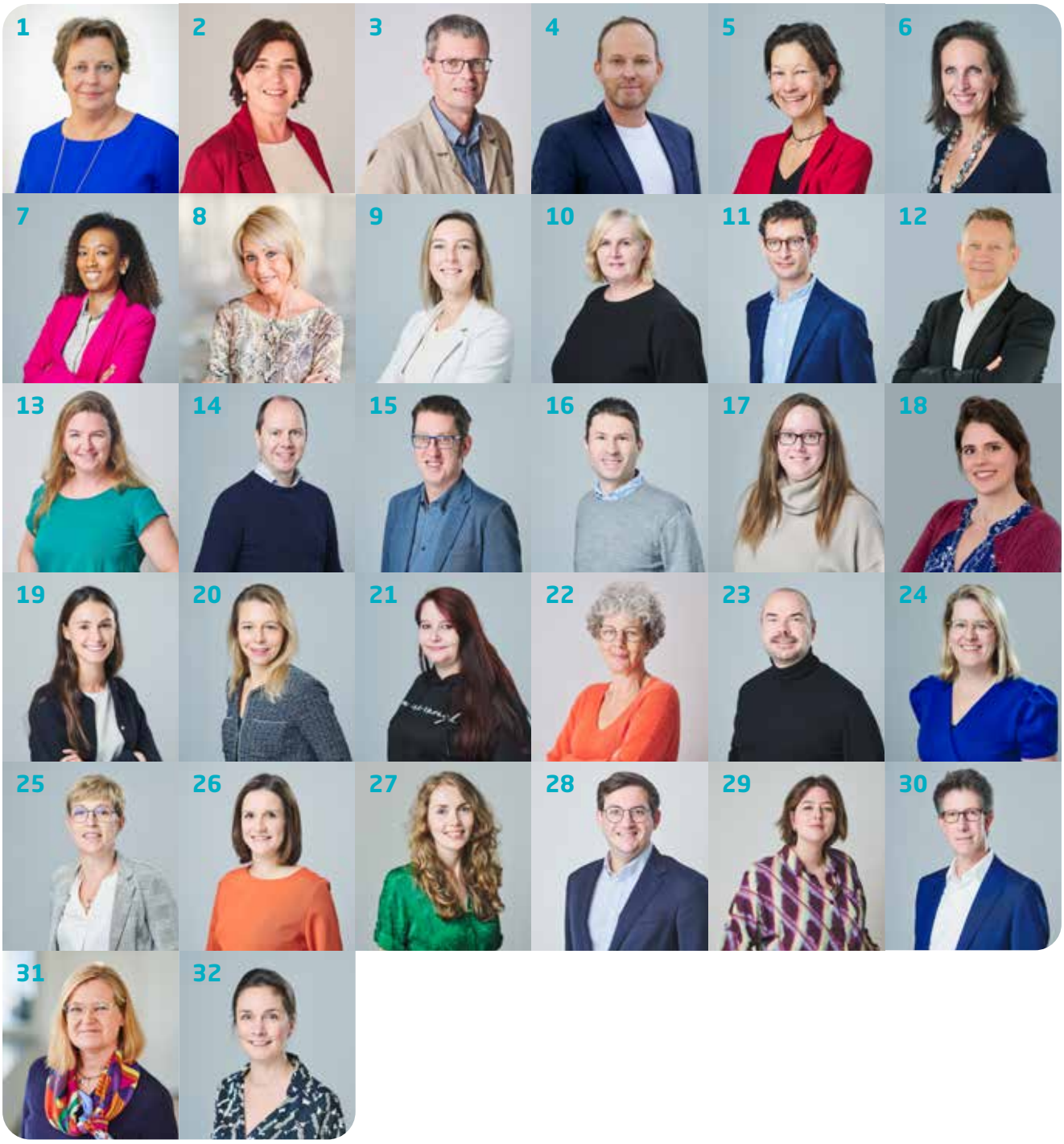
The Board of Directors is responsible for the strategic management of pharma.be and consists of **15 directors.** The current Chair of the Board of Directors is Frédéric Clais (Eli Lilly Benelux). Vice-chairman is Xavier Hormaechea (UCB). Board members' terms of office are valid for three years. They are elected at the general meeting.

1 Frédéric Clais Eli Lilly Benelux **Chair of pharma.be** **2 Xavier Hormaechea** UCB Pharma **Vice-chairman of pharma.be** **3 Renaud Decroix** AbbVie **4 Sara Schaufelberger** Amgen **5 Angela Thompson** AstraZeneca **6 Niels Hessmann** Bayer **7 Sally McNab** Bristol-Myers Squibb Belgium **8 Emmanuelle Boishardy** GlaxoSmithKline Pharmaceuticals **9 Marleen Hoebus** Janssen-Cilag **10 Katrien De Vos** MSD Belgium **11 Federico Mambretti** Novartis **12** Pfizer **13 Marie-José Borst** Roche **14 Bart Vermeulen** Sanofi Belgium **15 Paul Newton** Vertex Pharmaceuticals

1.6 With a team of experts

The pharma.be team consists of **32 highly motivated employees** with a variety of skills and expertise. They provide services to our member companies, represent them on relevant councils, committees, and advisory bodies, and promote their interests at various levels.

Meet the pharma.be team



1. Caroline Ven CEO **2. Ann Adriaensen** Secretary General & Public Health Director **3. Geert Steurs** Economics Director - Chief Economist **4. David Gering** Communications Director **5. Julie Gusman** Market Access Services Director **6. Patricia van Dijck** Political & Medical Director **7. Jennifer Andzouana** Members, Partners & Office Assistant (external) **8. Magali Audiart** Pricing & Market Access Advisor **9. Melanie Balcaen** Finance & HR Manager **10. Denise Blockmans** Webmaster & ICT Manager **11. Thomas Cloots** Economic Advisor **12. Willy Cnops** Life Science Advisor (external) **13. Karen Crabbé** Economic & Health Data Advisor **14. Guy De Backer** IT Consultant (external) **15. Johan De Haes** Public & Animal Health Advisor - SME Account Manager **16. Tom De Spiegelaere** Healthcare Budget Advisor **17. Anne-Sophie Doms** Content Manager **18. Lize Fonteyn** Market Access Advisor **19. Olivia Geldof** Legal Advisor **20. Nathalie Lambot** Public Health & Clinical Trials Advisor **21. Chloé Legrand** Members, Partners & Office Assistant **22. Nathalie Leroy** Personal Assistant to the CEO **23. Filipo Serra** Market Access Advisor **24. Annick Vancutsem** Members, Partners & Office Assistant **25. Carine Vancutsem** Members, Partners & Office Manager **26. Marie Vande Ginste** Prevention & Sustainability Advisor **27. Laura Van Eeckhout** Policy Advisor **28. Quentin Vanleeuw** Project & Process Manager **29. Oona Van Nieuwenhove** Public Health Advisor **30. Armand Voorschuur** European Policy and Market Access Advisor **31. Marjan Willaert** Policy Advisor – Market Access **32. Hanne Wouters** Market Access Advisor

1.7 Specialised partners

Biopharmaceutical knowledge and the regulatory framework are evolving at a rapid pace, leading to growing complexity. That is why, our members are increasingly looking for external expertise to keep up with the latest developments.

To enhance interaction between members and service providers, we developed modular partner services. In this way, we help create an active community, stimulate exchanges and networking between our members and partners, and strengthen the Belgian biopharmaceutical ecosystem.

As of 2024, pharma.be has established partnerships with 55 organisations across various fields, including pricing and reimbursement, medicine registration, pharmacovigilance, clinical trials, legislation, therapy compliance and proper use of medicines, and logistics.

Discover our partners here:

1.8 Internationally embedded

As an association, pharma.be is also embedded internationally, first and foremost as a member of the **European Federation of Pharmaceutical Industries and Associations (EFPIA).** EFPIA represents the biopharmaceutical industry operating in Europe. The direct members include 37 national associations, 38 leading biopharmaceutical companies and a growing number of small and medium-sized enterprises (SMEs). EFPIA's mission is to create a collaborative environment that enables its members to discover, develop and deliver new treatments and vaccines for people across Europe, as well as contribute to the European economy.

pharma.be is also a member of the **International Federation of Pharmaceutical Manufacturers and Associations (IFPMA)**, representing innovative biopharmaceutical companies and regional and national associations worldwide.

What we do 2

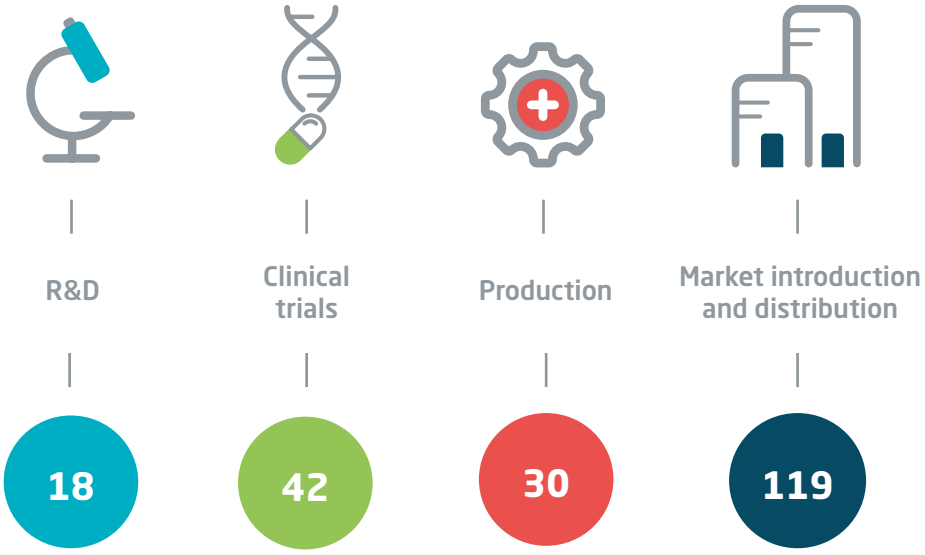
2.1 Operating across the entire value chain

Together, the pharma.be member companies **hold strong positions in Europe in every key aspect of the biopharmaceutical value chain**, from R&D and clinical trials over production to medicine marketing and distribution. This is due to a unique combination of a well-developed ecosystem, highly skilled workforce and strong cooperation with governments and research centres.

The benefits of an integrated approach to the value chain are great, both for patients, their environment and healthcare, and for the economy. This was abundantly clear during the COVID-19 pandemic. The Belgian biopharmaceutical sector was able to fully meet the challenge, from research and production to the introduction of innovative solutions for patients.

In this section, we delve into the innovation activities of our member companies in Belgium, focusing on delivering improved solutions for patients: the level of their R&D investments, the clinical trials they conduct here, the necessary focus on prevention, and the need for new antibiotics. We also provide an overview of the new medicines for which our companies have requested and received reimbursement and, finally, address the challenges surrounding rare diseases. In chapter three, we will discuss the added value of these activities for patients, the healthcare system and society, and their economic return.

Activities of pharma.be members in belgium



Source: pharma.be

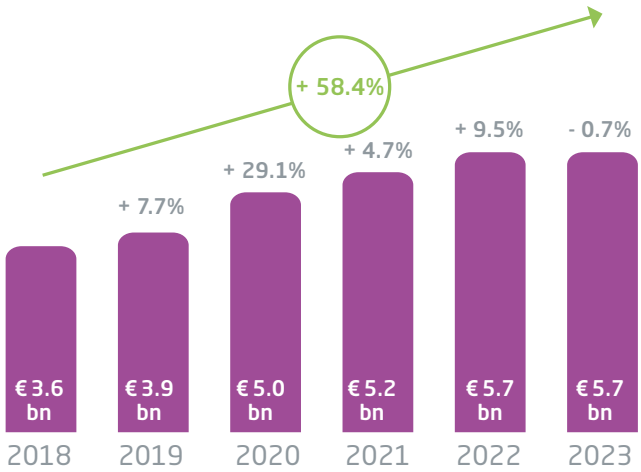
2.2 Research & Development as drivers of innovation

2.2.1 R&D investments in Belgium

What Belgium lacks in terms of natural resources, it makes up for in knowledge. Research and Development (R&D) is not only in Belgium's DNA, but it's also at the heart of the biopharmaceutical industry. The sector delivers a lot of important innovations every year. The added value is immediately visible, as we live longer and better thanks to these innovations.

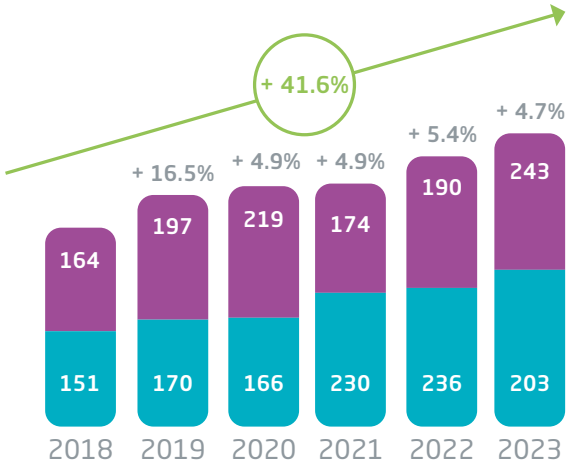
- In 2023, the sector invested **over 15 million euros in R&D every day**, amounting to a total of 5.7 billion euros.
- Over the past 25 years, investment in R&D has increased fivefold, and in just the past five years, it has risen by no less than 58%.
- In 2023, on average **more than one patent application was filed per day** in Belgium in the field of biotechnology and pharmaceuticals. These applications are crucial for the sector because the protection provided by patents is a prerequisite for continuing to invest in very expensive, long-term research programmes.
- Since 2018, the number of patent applications in these fields has increased by almost 42%, about 2.5 times higher than the growth in all technology domains combined. In 2023, for example, the sector accounted **for nearly 18% of the total number of patent applications** in Belgium. This makes the biopharmaceutical sector an absolute leader in innovation.

Evolution of R&D investment in Belgium (billion euros)



Source: pharma.be, survey amongst members

Evolution in patent applications in Belgium



Source: European Patent Office, European patent applications 2011-2021 per field of technology

Biotech Pharma

2.2.2 R&D investments compared to Europe

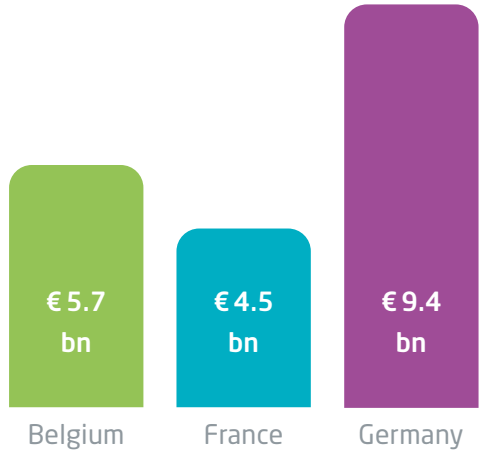
Although Belgium is a relatively small country, the people and the healthcare system can count on a proportionately high investment in biopharmaceutical innovation. **The total amount of R&D investment in the Belgian biopharmaceutical sector is comparable to that of much larger European countries.** In terms of R&D investments in 2022, the most recent year for which European comparisons are available, Belgium ranks just behind Germany, while surpassing France. The Belgian biopharmaceutical sector invested approximately the same amount as Italy, Denmark, Spain, and Sweden – ranks 4 to 7 – combined in absolute terms. An impressive tally, considering Belgium is eighth on the list of European countries by population.

Looking at investment per inhabitant, Belgium is definitely in the lead. Our investments in 2022 were almost two times higher than those of Denmark, second in the ranking, and were as much as almost three times higher than investments in Slovenia, number three in the ranking.

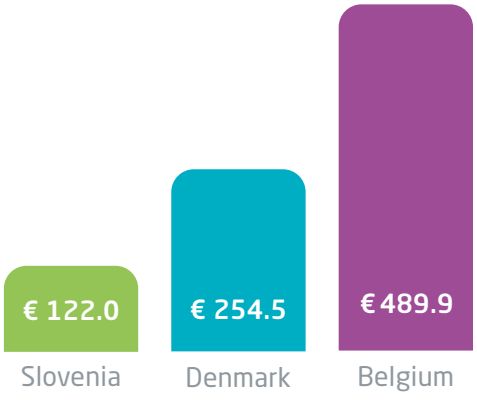
Over the period 2018-2022, Belgian R&D expenditures per capita grew by more than 56%, approximately 2.5 times the growth rate of total R&D expenditures per capita in the EU27. No other country with a significant amount of R&D expenditures per capita realized such strong growth. Denmark, in second place, actually showed significantly negative growth.

These impressive figures demonstrate that innovations cannot be taken for granted. New, innovative medicines require a particularly large amount of time and resources. The biopharmaceutical sector is therefore the most R&D-intensive industry. Not only in Belgium, but also in Europe, the Belgian biopharmaceutical sector is increasingly important in terms of R&D.

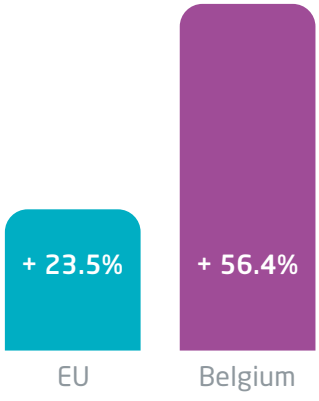
Total biopharmaceutical investments 2022



Biopharmaceutical investment in R&D per capita 2022



Growth of biopharmaceutical investment in R&D per capita 2018-2022



Sources: pharma.be, survey amongst members & EFPIA, The Pharmaceutical Industry in Figures, Key Data 2024

2.3 Clinical trials for new therapies

Clinical trials are crucial for the development of new medicines or vaccines. Through such trials, researchers can not only test whether a treatment is effective and safe but also gain valuable insights into the treatment or prevention of diseases. Clinical trials also offer patients free access to the latest treatments even before they reach the market. Therefore, a stimulating research environment is essential to achieve advancements in healthcare and health sciences.

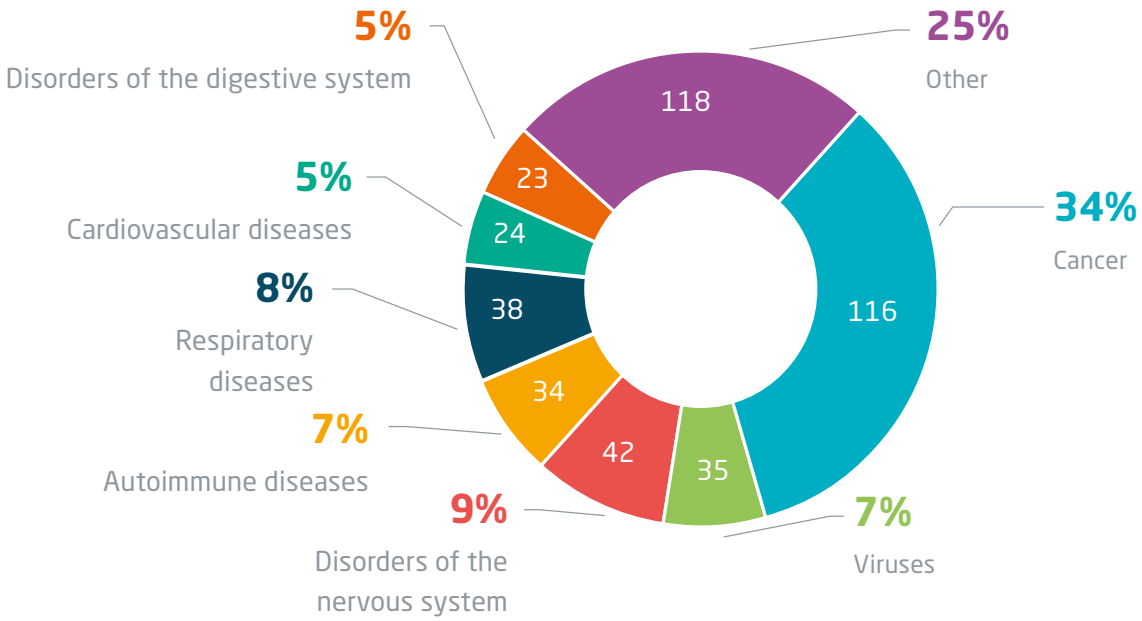
Belgium remains a leader in clinical research

Belgium boasts an impressive track record in clinical research. This success can be attributed to our dynamic ecosystem, supported by a strong presence and expertise of biopharmaceutical companies, the outstanding quality and knowledge of our academic community, state-of-the-art research centres and hospitals, and the in-depth expertise of relevant authorities, particularly the Federal Agency for Medicines and Health Products (FAMHP).

These factors enable Belgium to consistently rank among Europe's top countries in clinical trials. In 2023, this trend continued, with a total of 425 approved applications, over three-quarters of which came from the biopharmaceutical industry. Companies thus continue to systematically invest in developing new treatments across a wide range of therapeutic domains (see chart).

Cancer research ranks first among these, accounting for 34%. In 2022, Belgium initiated 161 clinical trials in this field, or approximately one-fifth of all cancer-related clinical research in Europe. This proportion clearly illustrates the impact of the Belgian biopharmaceutical industry within Europe in this area.

Share of clinical trial applications by disease domain



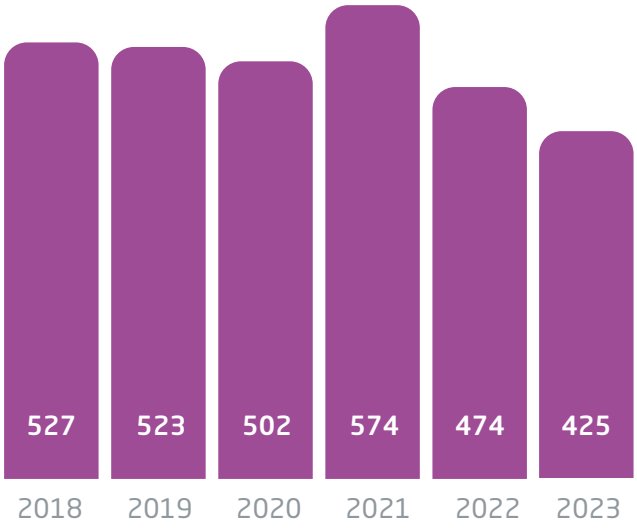
Source: Deloitte report "Belgium as clinical trial location in Europe - key results 2022"

Top position under pressure

However, our country's top position in clinical trials is under pressure, as evidenced by developments over the past eight years.

If the Belgian biopharmaceutical industry is to remain competitive on a global scale, our country must not only keep up with the latest technologies, research methodologies, and regulations but also help shape and lead them. Only by continuing to foster a stimulating research environment can we provide the best care for patients and society.

Number of approved applications for clinical trials



Source: FAMHP data

Decentralisation for a dynamic and innovative research environment

One of the developments closely followed by Belgian biopharmaceutical companies is the decentralisation of clinical trials. This involves conducting certain research activities, such as administering medication, follow-up conversations, or completing questionnaires, not in hospitals but in the patient's home environment, with consent, of course. This decentralisation is made possible by technological advancements, such as wearables (portable devices that monitor parameters like heart rate).

For patients, this can provide significant benefits: they don't need to travel as often and can remain in the comfort of their own homes. For researchers, it offers considerable advantages too, allowing better monitoring of participants and providing more reliable, real-time data in real-life settings.

Biopharmaceutical companies are eager to support this trend towards decentralisation, enhancing patient convenience and strengthening the quality of trials. **Therefore, pharma.be advocates for dialogue and collaboration with all relevant partners to implement further decentralisation**. This approach helps Belgium maintain its international reputation as an innovative, high-quality, and flexible environment for clinical research.

pharma.be consults patient organisations on home-based clinical trials

Given the potential added value of decentralisation for patients, pharma.be conducted a survey in 2024 involving 31 patient organisations. The survey explored attitudes and willingness regarding remote (follow-up) visits, home visits by nurses, and the use of digital tools to collect real-time data. Patients could also answer open questions about what they see as the potential pros and cons of decentralisation and which research elements could be organised remotely.

Some key findings from the survey:

- Patients indicated that decentralisation could increase their motivation to participate in a clinical trial, as it saves time and reduces the overall impact on their daily lives.
- Decentralisation may also improve treatment adherence, as online visits and digital tools offer more flexibility and convenience in care.
- More than four out of five respondents believed decentralisation could also help increase diversity in clinical trial participation, though they didn't think decentralisation alone could achieve this.

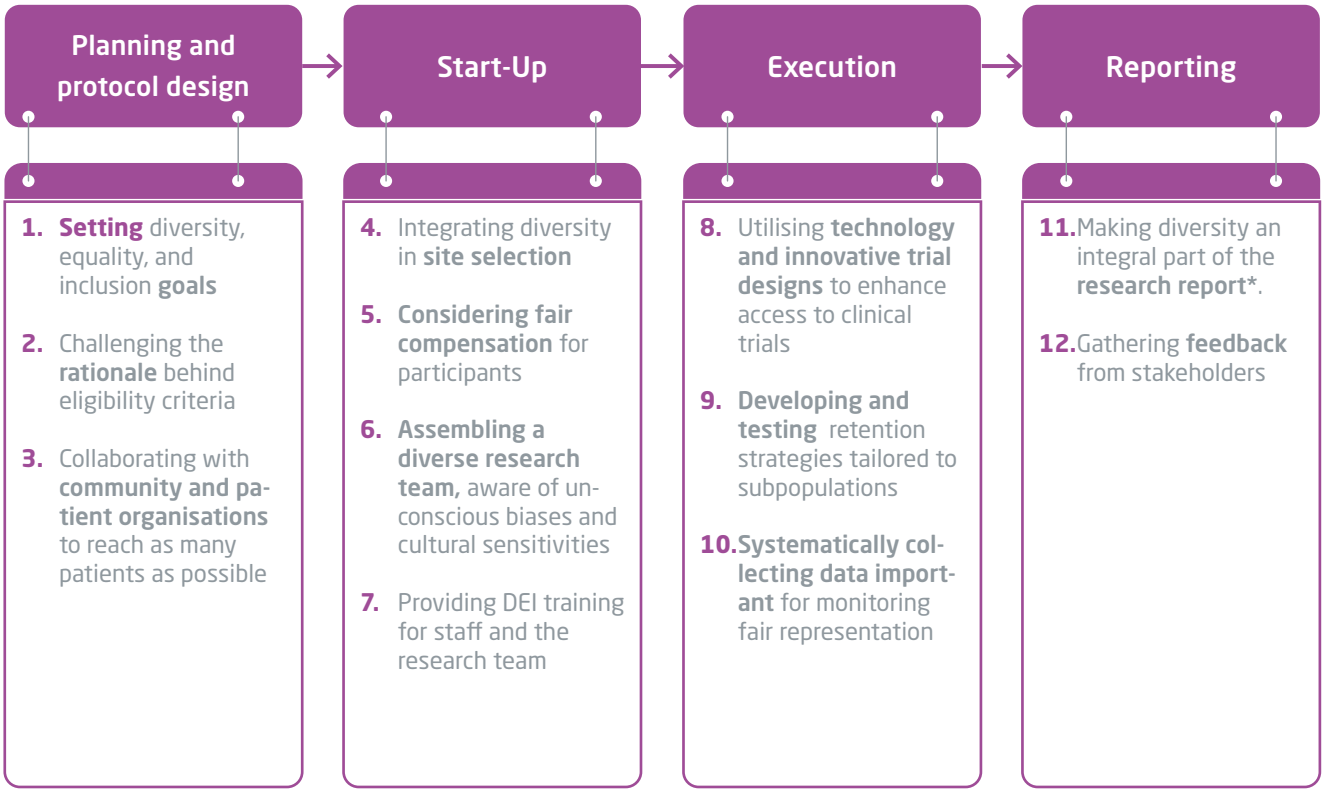
Overall, the survey showed that patients see primarily positive aspects in decentralisation and are keen to embrace such an approach. However, some potential drawbacks and concerns were also noted, such as reduced contact with the research team and the risk of loneliness or diminished trust. It's also essential to ensure that using digital tools does not raise, rather than lower, the barrier to participation.

You can read the full results of the survey here:

Inclusion and diversity must remain ensured

pharma.be not only advocates for retaining and strengthening our leading position in clinical trials but also strives to make these trials easily accessible to a diverse population. Only in this way can trials yield results that are generalisable and applicable to all who might benefit.

It is essential that clinical trial participants come from varied backgrounds and characteristics, such as age, gender, ethnicity, and socioeconomic status. While this may seem straightforward, it presents real challenges in practice. **To address these issues, pharma.be dedicated its annual Clinical Trial Forum in early 2024 to the theme of "embracing diversity & equity in clinical trials".** Deloitte presented twelve recommendations to enhance diversity and inclusion in clinical research in Belgium.



**As part of current good clinical practice, a demographic breakdown of the study population is included in the research report.*

Source: Deloitte report "Belgium as clinical trial location in Europe - key results 2022"

Belgium remains a leader in clinical trials per capita in Europe, with 474 authorized in 2022, 80% initiated by companies. At Eli Lilly, we proudly contribute by conducting about 35 trials, offering 800 patients early access to innovative treatments. To secure Belgium's future as a top research hub, we urge policymakers to adopt flexible (decentralized) trials and enhance real-world data use. These measures would boost participation, improve diversity, and enhance both patient and trial outcomes.

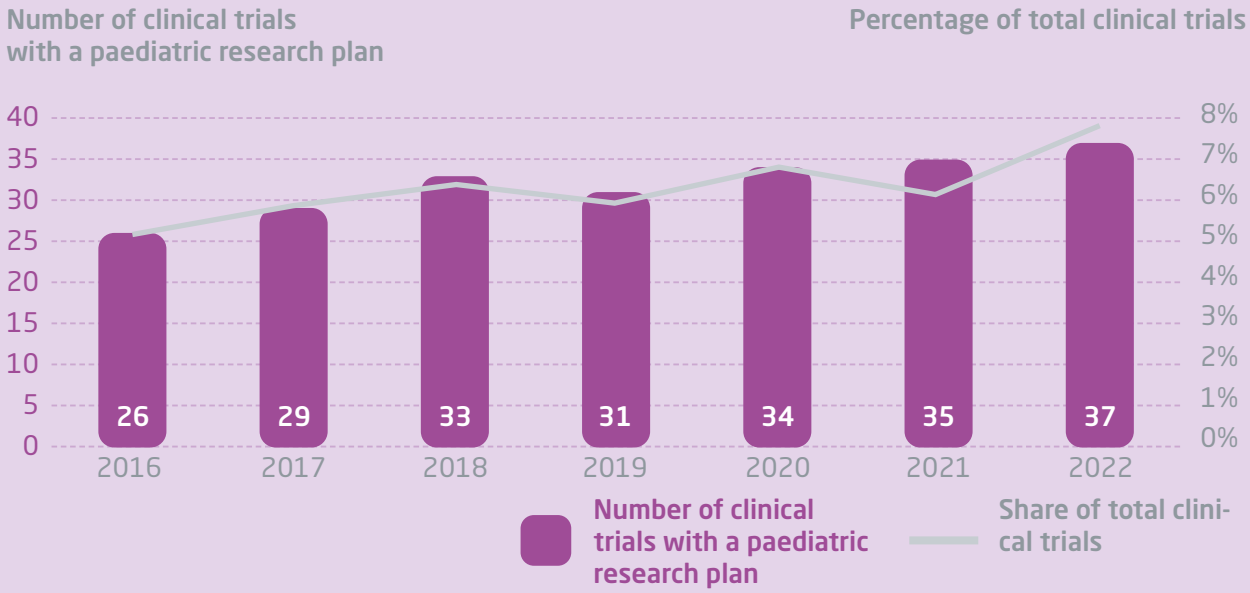
Ana Carla Franch, *Director, Investigator Engagement, Clinical Research Investigator Engagement, Delivery & Capabilities S.A. Eli Lilly Benelux N.V.*

Paediatric clinical trials: a specific and highly relevant form of clinical research

Belgium's impact on paediatric clinical trials within Europe continues to grow.

In 2022, Belgium accounted for 15% of the total in Europe.

Evolution of clinical trials with a paediatric research plan in Belgium (2016-2022)



Source: Deloitte report "Belgium as clinical trial location in Europe - key results 2022"

Paediatric clinical trials are essential to ensure that this group has access to new medicines tailored to their anatomy and physiology. Children are not merely "small adults"; a simple dosage adjustment does not suffice. Since 2007, the European Medicines Agency (EMA) has required that any application for the registration of a new medicine or indication includes a plan for paediatric research.[1] This allows for examining if and how the medicine can be used in children. By July 2024, the EMA had assessed a total of 2,871 such plans from biopharmaceutical companies since this requirement was introduced.

Paediatric clinical trials present unique challenges. They must be conducted by highly qualified professionals with expertise in paediatrics. Additionally, a strong network of research centres is essential to facilitate knowledge-sharing among researchers, streamline trial coordination, and effectively raise awareness and inform about clinical research opportunities for children in Belgium.

To further enhance Belgium's positive impact in this field, initiatives such as the *Antwerp Pediatric Clinical Trial Network are essential.*[2] Supported by various partners from the biopharmaceutical industry, it focuses on coordinated efforts in vaccine research and infectious diseases in children. The Belgian Pediatric Clinical Research Network (BPCRN) is another significant initiative. It was launched by the Belgian Society of Pediatrics in 2009 with the support of pharma.be and was revitalised in 2024 under the auspices of the *Belgian Academy of Paediatrics.*[3]

A new brochure on clinical trials to raise patient awareness

To ensure patients gain timely access to the latest medicines, accurate and clear information about clinical trials is a critical first step.

What is a clinical trial? Who is involved? How can I participate? What does participation entail? What data is collected, and how is it used? These are just some of the questions for which patients often struggle to find straightforward answers.

Therefore, pharma.be developed a new brochure in collaboration with representatives from patient organisations. This clear and accessible resource provides patients and healthcare providers with a comprehensive tool to address the subject.

Access the brochure:

Our ELECTS center in Wallonia plays a vital role in supporting Eli Lilly's global clinical trials, coordinating the packaging and distribution of materials to EMEA and Asia Pacific. This ensures trial sites worldwide receive resources efficiently, accelerating research and patient access to new medicines. As we innovate, decentralizing trials will be crucial to meeting patient needs and maintaining Belgium's competitive edge in clinical research.

Benoit Lessire, *Senior Director, ELECTS Site Head and Global Leader-CT Material Logistics, Eli Lilly European Clinical Trial Services S.A.*

2.4 Prevention as a key to a healthy future

Prevention involves proactive measures that promote health and prevent or slow the onset, spread, and progression of diseases. These measures can include vaccines, predictive AI algorithms, and efforts to improve air quality.

The biopharmaceutical sector strongly prioritises prevention, as it improves population health, helps reduce health inequalities, and lessens the strain on our healthcare system. This approach paves the way for socially, economically, and ecologically sustainable care in Belgium.

Vaccination as a form of prevention

A particularly effective form of prevention is vaccination. It protects not only individuals but also entire communities by building herd immunity. Basic vaccination programmes, for example, have significantly reduced childhood mortality rates worldwide. Smallpox, a severe disease, was eradicated globally in 1980, and polio has been eliminated from Europe thanks to vaccination.

Belgium is a leader in vaccine research and development, production, and export. From 2014 to 2023, Belgium contributed an average of 42.6% of the total EU export of vaccines for humans. The vaccination rate among children in Belgium is high, with over 95% receiving at least one vaccination against diseases such as measles, polio, and whooping cough.[4] However, there is room for improvement among adolescents and adults.

Immunisation across all life stages

The biopharmaceutical industry is prioritising efforts to encourage immunisation at every stage of life. To support this goal, pharma.be has mapped the challenges in Belgium's vaccination landscape through dialogue with various stakeholders and developed an initial list of policy proposals, later refined in collaboration with experts. This aims to help pharma.be shape a policy framework that supports immunisation across all life stages.

Looking back at the event "Data driven life course immunisation : What are we waiting for?"

In April 2024, Vaccines Europe and pharma.be hosted an event on data-driven immunisation across all life stages at Vaccinopolis (University of Antwerp). Scientists, industry representatives, and policymakers debated the importance of integrated data collection for an effective vaccination policy. Better data collection enables the assessment of disease burden, the identification and prevention of outbreaks, and the evaluation of vaccination programmes. For the industry, data collection is vital for monitoring vaccine effectiveness and advancing research and development of new or improved vaccines.

A highlight was the keynote by Professor Plotkin (USA), widely recognised as the "godfather of vaccines." He took attendees through the history of vaccines and shared his vision for the future of vaccine development. During the panel discussion, experts delved into the challenges and opportunities of data-driven immunisation, covering topics from the importance of collaboration and data analysis to the use of vaccination registries and the need for ongoing innovation in vaccine research.

One of the key conclusions was that a robust and integrated approach to data collection and analysis is essential for optimising vaccination programmes.

More Information:

2.5 The urgent need for new antibiotics

Since their breakthrough during WWII, antibiotics have become indispensable in our healthcare. However, if we fail to halt the rise of antibiotic resistance, we may face a future where doctors must manage without these essential medicines.

Antibiotics are commonly associated with fighting infections such as pneumonia. However, they have far broader applications, reducing risks during surgery and protecting cancer patients undergoing chemotherapy. Modern healthcare without antibiotics is nearly unthinkable.

Yet, with the rise of bacteria resistant to our antibiotics, these medicines are losing their effectiveness. Europe rightly considers antibiotic resistance as one of the top three health threats. The impact of antibiotic resistance is increasingly evident in Belgium, where Sciensano reports that 7% of hospital patients acquire infections with resistant bacteria, leading to approximately 2,600 deaths each year.

The AMR Action Fund (https://www.amractionfund.com/) plays a vital role in the global effort to tackle antimicrobial resistance (AMR) and supports the development of new antibiotics in various ways.

Launched in 2020 by a group of biopharmaceutical companies, including pharma.be member companies, in collaboration with the World Health Organization (WHO), the European Investment Bank (EIB), and other partners, the fund aims to accelerate the development of new antibiotics, with the goal of bringing two to four new antibiotics to market by 2030.

While the AMR Action Fund is a significant step in combating antibiotic resistance, it is only part of the solution in developing new antibiotics.

Creating new antibiotics is crucial for both individual patients and the population as a whole. It helps combat antibiotic-resistant bacteria, protects vulnerable groups, supports medical treatments, prevents infection spread, and contributes to economic benefits and a stronger healthcare system. By investing in new antibiotics, we can prepare for future health challenges and improve public health worldwide.

2.6 Newly reimbursed medicines

The R&D activities and clinical trials conducted by our member companies in Belgium and abroad result in the development of new medicines. To make these available to patients in Belgium, companies apply for reimbursement with the National Institute for Health and Disability Insurance (NIHDI) so that patients do not have to pay the full cost.

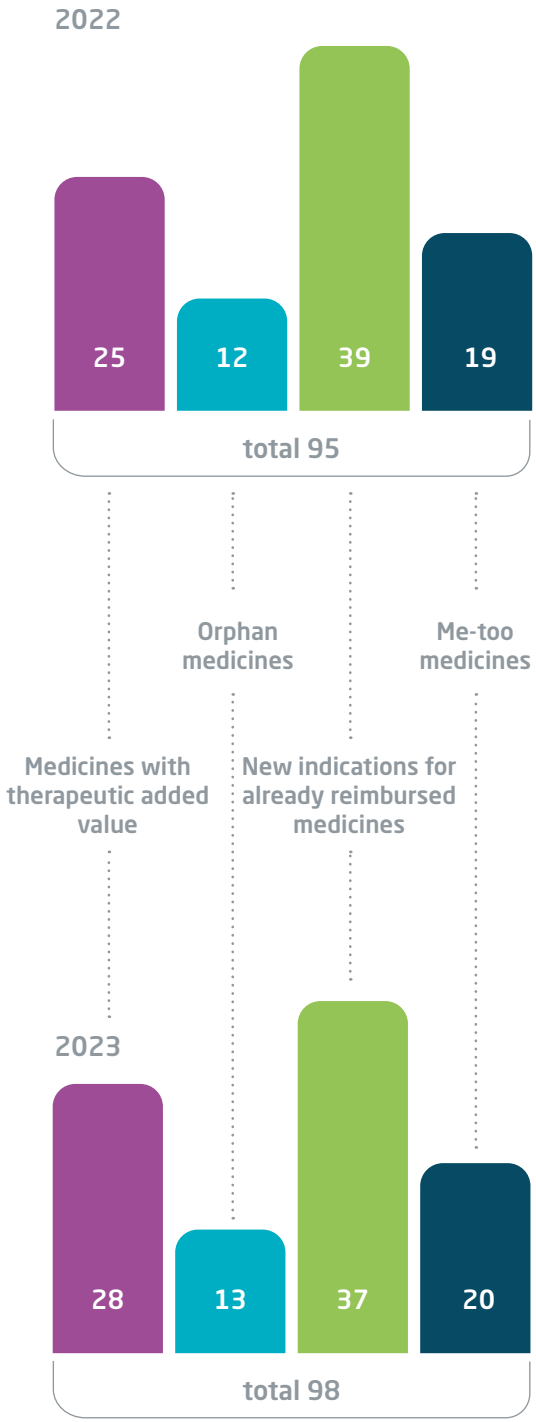
Below we provide information on the number of newly reimbursed medicines in Belgium in 2023. The added value of some of these medicines is discussed in chapter three.

2.6.1 According to type

In 2023, **98 new medicines or indications** were reimbursed (see figure for distribution by type).

Notes:

- A medicine **with therapeutic added value offers higher therapeutic value** than an accepted standard treatment, according to the biopharmaceutical company concerned. This means the disease is better treated with this medicine.
- An **orphan medicine** is a medicine for the treatment of a rare disease and therefore often provides a solution to an unmet medical need.
- A **new indication** refers to a medicine that is already reimbursed for a particular indication/condition and for which the company applies for additional reimbursement for another indication/condition. For example, this could be a medicine that is already reimbursed for the treatment of lung cancer but is now also reimbursed for the treatment of colon cancer.
- A me-too medicine **does not** offer a higher therapeutic value than existing medicines for the same indication/disease but may add value to the patient because of improvements in dosage, administration schedule, comfort or ease of use. An additional advantage of these medicines is the greater guarantee that a treatment can be continued in the case of unavailability of the existing medicines.



2.6.2 According to therapeutic area

The table below provides an overview of the therapeutic areas (Anatomical Therapeutic Chemical, ATC) for which new medicines were reimbursed in 2023. More than half of these were new cancer medicines or immunomodulatory agents (ATC L). In addition, relatively more new medicines have become available for dermatological (ATC D), infectious (ATC J), neurological (ATC N), and respiratory diseases (ATC R).

ATC code	Number of newly reimbursed medicines in 2023	ATC main group	Examples of pathology
A	5	Alimentary tract and metabolism	Pompe disease, primary hyperoxaluria type 1
B	3	Blood and blood-forming organs	Acquired thrombotic thrombocytopenic purpura
C	2	Cardiovascular system	Chronic kidney disease, familial hypercholesterolemia
D	8	Dermatology	Atopic dermatitis, actinic keratosis
G	0	Genitourinary system and sex hormones	-
H	2	Systemic hormonal preparations (excluding sex hormones and insulins)	Acromegaly
J	7	Anti-infectives for systemic use	COVID-19, pneumococcal infections, hepatitis B, varicella zoster
L	53	Antineoplastic and immunomodulating agents	Colorectal cancer, lung cancer, breast cancer, kidney cancer, urothelial cancer, uveal melanoma, mastocytosis, chronic myeloid leukaemia, graft-versus-host disease, myelofibrosis, chronic lymphocytic leukaemia, diffuse large B-cell lymphoma
M	2	Musculoskeletal system	Urinary incontinence
N	7	Nervous system	Pain, epilepsy, apnoea in premature babies, aromatic L-amino acid decarboxylase (AADC) feficiency
P	0	Antiparasitic products, insecticides and repellents	-
R	7	Respiratory system	Asthma, cystic fibrosis
S	2	Sensory organs	Age-related macular degeneration, (diabetic) macular oedema
V	0	miscellaneous	-

2.7 Rare diseases: challenges and solutions

While each rare disease affects relatively few people, in total **more than half a million Belgians** have a rare disease. Despite significant healthcare breakthroughs in recent decades, many rare diseases remain difficult or even impossible to treat. pharma.be supports the federal government's efforts to develop a new Belgian plan for Rare Diseases.

Rare diseases, but many patients

A disease is considered rare if it affects no more than 1 in 2,000 people in the European Union. In fact, 84% of all known rare diseases affect fewer than 1 in 1 million people. However, despite the small number of patients per rare disease, it is estimated that 300 million people worldwide have a rare disease. In Belgium, this figure stands at over half a million. This is because there are so many rare diseases—currently, around 7,000 are known globally.[5]

Unique challenges

Developing effective medicines for rare diseases is not straightforward. Many rare diseases still lack basic scientific knowledge regarding their origins and progression. Even if a promising molecule is identified that could potentially cure or slow down a rare disease, further development is particularly challenging. For instance, organising clinical trials – a crucial phase in developing new medicines – is difficult due to the limited number of patients, who are often scattered across the globe.

New treatments

The pharmaceutical industry continues to invest in research and development of orphan medicines. In 2022, 24% of approved clinical trials in Belgium focused on rare diseases, totalling 122 new trials. This research produces pharmaceutical products to diagnose, prevent or treat rare diseases with no existing alternatives.[6] Between 2003 and 2023, the *European Medicines Agency* (EMA) approved over 240 of these orphan medicines.

3

For whom we create added value

In this chapter, we will first delve into the added value created by the biopharmaceutical sector for patients, the healthcare sector, and society. pharma.be compiled a comprehensive list of scientific studies addressing the added value of innovative medicines and vaccines. We have summarised several striking examples. We illustrate the added value of innovative medicines with some examples of medications newly reimbursed in 2023 and also look ahead to radioligand therapy, a groundbreaking cancer treatment.

However, the story does not end there. Our sector is also an important driver of the knowledge economy. Employment and export were already impressive and have only continued to grow in 2023, as is reflected in the positive cost-benefit analysis of our sector for the Belgian government.

3.1 A positive impact for patients, healthcare sector and society

3.1.1 The societal impact of innovative medicines cannot be underestimated

Every day, medicines prove their ability to cure people and improve quality of life. Yet, their added value and impact reach much further. Numerous studies consistently highlight this, making it essential to map these impacts. Solid data reveal significant positive effects on life expectancy, hospitalisation rates, and productivity. In the following sections, we illustrate these findings with clear figures.

Medicines help us live longer. Innovative medicines have increased our life expectancy, transforming previously fatal diseases into manageable chronic conditions and significantly preventing premature death.

In high-income countries, including Belgium, innovative medicines contributed 73% to the increase in average life expectancy between 2006 and 2016, adding 1.23 years.[7]

Expressed differently, pharmaceutical innovation has reduced premature mortality in Belgium by 22% since 2000.[8]

We spend less time in hospital. Innovative medicines reduce the pressure on healthcare systems by preventing complications and reducing hospital admissions, making resources significantly more efficient.

Pharmaceutical innovation in Europe saved 13 billion euros in healthcare costs by avoiding complications between 2007 and 2017.[9]

A study in 15 OECD countries estimated that without the introduction of new medicines after 1981, people would stay in hospitals more often and for longer. From 1982 to 2015, new medicines resulted in 91% more hospital discharges and 163% fewer hospital days.[10]

We can reintegrate into society more quickly. Innovative medicines enable people to work, study, or care for their families, contributing to society. Many studies quantify this impact in monetary terms, providing numerous compelling examples.

Overall, innovative medicines have delivered 27 billion euros in productivity gains to EU economies. Specific innovations in HIV treatment have proven highly impactful, with 22 billion euros in productivity gains between 2007 and 2017.[11]

For melanoma patients, immuno- and targeted therapies have drastically improved survival rates, yielding an estimated 3.8 million work hours and 391 million euros in annual income across the EU27 due to improved productivity from 2011 to 2020.[12]

In Belgium, health-economic analyses show that an average of 3,869 euros was gained per patient per year between 2000 and 2017.[13]

We live longer

+1.23 years
(2006-2016)

Spend less time in hospital

-22% early mortality
(since 2000)

Reintegrate more quickly into society

€3,869 health gain
per patient per year
(2000-2017)

3.1.2 Added value of newly reimbursed medicines

In 2023, many new medicines were approved for reimbursement (see 2.6), offering significant added value for patients. Below, we provide **examples** from the group of **medicines whose therapeutic benefits were recognised by the Commission for Reimbursement of Medicines (CRM), including orphan medicines.**

Aromatic L-Amino Acid Decarboxylase (AADC) Deficiency

Children with AADC deficiency produce insufficient aromatic L-amino acid decarboxylase (AADC), triggering a chain reaction: AADC is essential for producing dopamine and serotonin, which in turn are crucial for making (nor) adrenaline.[14, 15] A lack of AADC results in a range of symptoms, including movement disorders, developmental delays, and poor functioning of the autonomic nervous system.

Since 2023, a new gene therapy for treating AADC deficiency has been reimbursed in Belgium.[16] This **one-time gene replacement therapy addresses** the root cause of the disease by **restoring dopamine production in the brain**, leading to lasting neurological and neuromuscular improvements.[17] This breakthrough offers hope for treating this fatal, rare genetic childhood disease that causes severe disability and suffering from the first months of life.

COVID-19

Around 80% of people infected with COVID-19 experience mild or no symptoms. However, those with compromised immunity, particularly the elderly or chronically ill, may develop severe pneumonia, which can be fatal.[18, 19, 20] While vaccination generally provides protection, its effectiveness wanes over time and may not be optimised for new virus variants.[21]

Antiviral treatments that inhibit viral replication in the body are thus essential in combating COVID-19. **The first oral antiviral COVID-19 medicine, a protease inhibitor, has been reimbursed in Belgium since 2023 for patients not requiring supplemental oxygen but at higher risk of severe COVID-19.[22] Administered orally, the treatment can begin promptly without hospital admission.[23]**

Taken within five days of initial symptoms, these tablets quickly reduce the viral load, lowering the risk of hospitalisation and death from COVID-19. Patients requiring admission experience shorter stays and/or less intensive care, with fewer medical consultations overall.[24] **The medicine is effective against all known virus variants.**[25, 26, 27, 28]

HER2-Positive Breast Cancer

Each year, over 530,000 people in Europe are diagnosed with breast cancer, and approximately one in five has HER2-positive breast cancer, typically associated with aggressive tumours and a poorer prognosis.[29, 30, 31]

Since July 2023, a new medicine has been reimbursed in Belgium for treating adult patients with unresectable or metastatic HER2-positive breast cancer who have already received one or more anti-HER2 treatments.

This new HER2-targeted medicine utilises a unique antibody-drug conjugate (ADC) technology. It reduces the risk of disease progression and death by up to 72% compared to another ADC in patients previously treated with another HER2-targeted medicine and chemotherapy.

This new medicine can thus significantly enhance treatment options for patients with HER2-positive cancer.

HER2-positive breast cancer once had the poorest prognosis. Fortunately, thanks to continuous innovations over the past 20 years, we have already been able to improve this prognosis considerably. With new ADCs and tyrosine kinase inhibitors, we can now make a significant leap forward. This breakthrough gives me hope that in the future we may even be able to cure patients with metastatic HER2-positive breast cancer.

***Professor Dr. François Duhoux**, Oncologist at*
King Albert II Institute, Cliniques Universitaires Saint-Luc

High Immunity in Kidney Transplants

In Europe and the USA, around 170,000 patients with end-stage renal disease are on waiting lists for kidney transplants.[32] Finding a suitable donor is challenging, but for those with high levels of antibodies, the chance of a good match is even smaller. Due to previous blood transfusions, pregnancies, or transplants, these patients have many more antibodies, increasing the risk of rejection.[33, 34, 35, 36, 37] While awaiting a suitable kidney, they are often on long-term dialysis, which can lead to complications, higher mortality risk, poorer quality of life, and increased costs.[38, 39]

Recently, a new, innovative enzyme therapy for desensitisation has been reimbursed. This treatment temporarily deactivates antibodies for a few days, reducing the risk of acute rejection of a new kidney, making transplantation feasible even with a less-than-perfect match.[40] Furthermore, the therapy shows positive long-term outcomes comparable to those of compatible kidney transplants.[41, 42]

This therapy provides new opportunities for highly sensitized patients, reducing wait times and eliminating the need for long-term dialysis. Consequently, survival rates and quality of life improve, while the therapy also contributes to better transplant care and relieves financial pressure on the healthcare system.

Multiple Myeloma

Multiple myeloma is a type of bone marrow cancer. It is diagnosed in approximately 1,000 Belgians each year.[43] Patients ineligible for bone marrow transplants have an average life expectancy of 3.5 to 6 years.[44] Fortunately, treatment options have increased significantly in recent years.

Since 2023, a medicine with a new mechanism of action is reimbursed for patients who have been treated with at least three other types of medicines and whose disease no longer responds to the latest treatment.

This new medicine brings T-cells – white blood cells in the body's immune system – into proximity with cancer cells, enabling the immune system to destroy the cancer cells.

This new treatment doubles the chance of a positive response and reduces the risk of death by 45%.[45] Quality of life also improves significantly.[46] Administered via a subcutaneous injection, this treatment requires less hospital time, and at-home administration is expected to become possible.[47, 48]

Renal Cell Carcinoma

Since 2023, patients with previously untreated advanced-stage kidney cancer have access to a new combination therapy comprising immunotherapy and a tyrosine kinase inhibitor that inhibits cancer cell division. This combination provides rapid, profound, and lasting results,

not only in clear cell kidney cancer – the most common form – but also in patients with non-clear cell kidney cancer, a type that is even more challenging to treat.[49, 50] **This highly effective new combination therapy adds a valuable first-line treatment option for kidney cancer patients.[51]**

Triple-Negative Breast Cancer

About 10% of all new breast cancer cases in Belgium are triple-negative breast cancers (TNBC).[52] TNBC is an aggressive form of breast cancer that is challenging to treat due to faster growth and malignancy.[53, 54, 55] Many patients, often younger women, experience relapse with an even more aggressive progression.[56] The 5-year relative survival rate for patients with metastatic TNBC is only 12%.[57]

Developing new and effective TNBC treatments is therefore essential for slowing disease progression, extending life, and improving quality of life. Since 2023, a medicine that represents a significant breakthrough has been reimbursed. It combines the specificity of an antibody targeting trophoblast cell surface antigen 2 (TROP2) with a cell-killing agent. It consists of an antibody that can recognise TROP2 (trophoblast cell surface antigen) on the surface of cancer cells, linked to a cell-killing agent.

This treatment has a manageable safety profile, enhances both survival and quality of life, and slows disease progression, providing a new treatment option for TNBC patients with significant medical and social benefits.

Urothelial Carcinoma

Urothelial carcinoma (UC) is a malignant tumour that forms on the inner lining of the bladder and urinary tract, causing urinary discomfort, lower back/abdominal pain, fatigue, weight loss, and loss of appetite.[58, 59] Advanced UC patients are initially treated with platinum-based chemotherapy and immunotherapy. When the disease progresses, however, treatment options become limited. Prognosis is poor, with around half of advanced UC patients dying within one year of diagnosis.[60]

Since March 2023, a new treatment has been available in Belgium for adult patients with advanced UC who have already undergone platinum-based chemotherapy and immunotherapy. This medicine employs an innovative, targeted mechanism, binding directly to a specific protein on the cancer cell surface, initiating cell-killing therapy, halting the cell cycle, and ultimately eradicating the cancer cells.[61]

This new medicine provides patients with advanced UC an average of four additional months of life without compromising quality.[62, 63]

Uveal Melanoma

Since November 2023, a treatment has been reimbursed in Belgium for the first time for adult patients with an inoperable or metastatic tumour in the eye (uveal melanoma or UM).[64] **UM is very rare; in Belgium, only about 50 people receive this diagnosis each year. Half of these patients develop metastases, primarily in the liver, with a life expectancy of approximately one year.[65]** To somewhat increase life expectancy, treatments for skin cancer have been used so far, even though they are not very effective for UM.[66]

The new medicine changes this. It is an immunotherapy based on a new technology, ImmTAC, which enables T-cells or immune cells to recognise specific UM cells and eliminate them. Thanks to this new therapy, the risk of death is reduced by nearly half, and average life expectancy rises to 22 months. Approximately one-quarter of patients experience a lasting positive effect. Side effects (such as rash, fever, low blood pressure), some of which can be severe, usually occur within the first month and improve over time.[67]

3.1.3 Radioligand therapy: a groundbreaking cancer treatment

With advancements in research and technology, the landscape of cancer treatment has witnessed significant progress, offering a range of therapeutic options: from traditional approaches like surgery, chemotherapy, and radiotherapy to more recent innovations like immuno-oncology medicines and now radioligand therapy (RLT). **Radioligand therapy is a pioneering and targeted treatment that uses nuclear medicine to treat various types of cancer with precision. It combines a molecule that recognises cancer cells (the ligand) with a radioisotope, aiming to damage or destroy cancer cells while minimising the impact on nearby healthy cells.** RLT has the potential to provide personalised and targeted treatments to cancer patients at an earlier stage of their disease by focusing on the unique characteristics of the cancer being treated, enhancing the effectiveness of the treatment.

Belgium has extensive experience in nuclear medicine and radioligand therapy. Our country has a unique ecosystem comprising leading nuclear experts, research facilities, research reactors, biopharmaceutical companies, production facilities, specialised physicians, and hospitals. In total, Belgium's nuclear medicine sector includes over 5,000 dedicated professionals, including 350 specialists in the field. With this wealth of expertise, Belgium can play a leading role in implementing current and future radioligand therapies, potentially becoming a global RLT hub and providing more patients in Belgium with access to groundbreaking treatments.

To sustain Belgium's leadership in RLT and make our healthcare system RLT-ready for the future, pharma.be is participating in a comprehensive 'RLT Action Plan' involving all relevant stakeholders, such as healthcare professionals, hospitals, research institutions, universities, companies, patient organisations, and supporting administrations (NIHDI, FAMHP, and FANC). Implementation of the 'RLT Action Plan' will commence in the coming year.

3.2 A positive impact on the economy

Belgium's unique *biopharma valley* has world-class players and is a leader in the development of revolutionary medicines and vaccines. **Besides added value for patients, the healthcare sector and society, our strong biopharmaceutical sector also provides a direct economic return and even a positive impact on public finances.**

In terms of employment, almost 142,000 jobs are linked to the biopharmaceutical industry (direct, indirect and induced employment). The sector also occupies an important place in terms of exports, contributing significantly to Belgium's positive trade balance. In addition, the huge investments in R&D and clinical trials (see above) make an important contribution to the Belgian knowledge economy, which is the basis of our welfare state.

3.2.1 The economic value of the biopharmaceutical sector

EMPLOYMENT

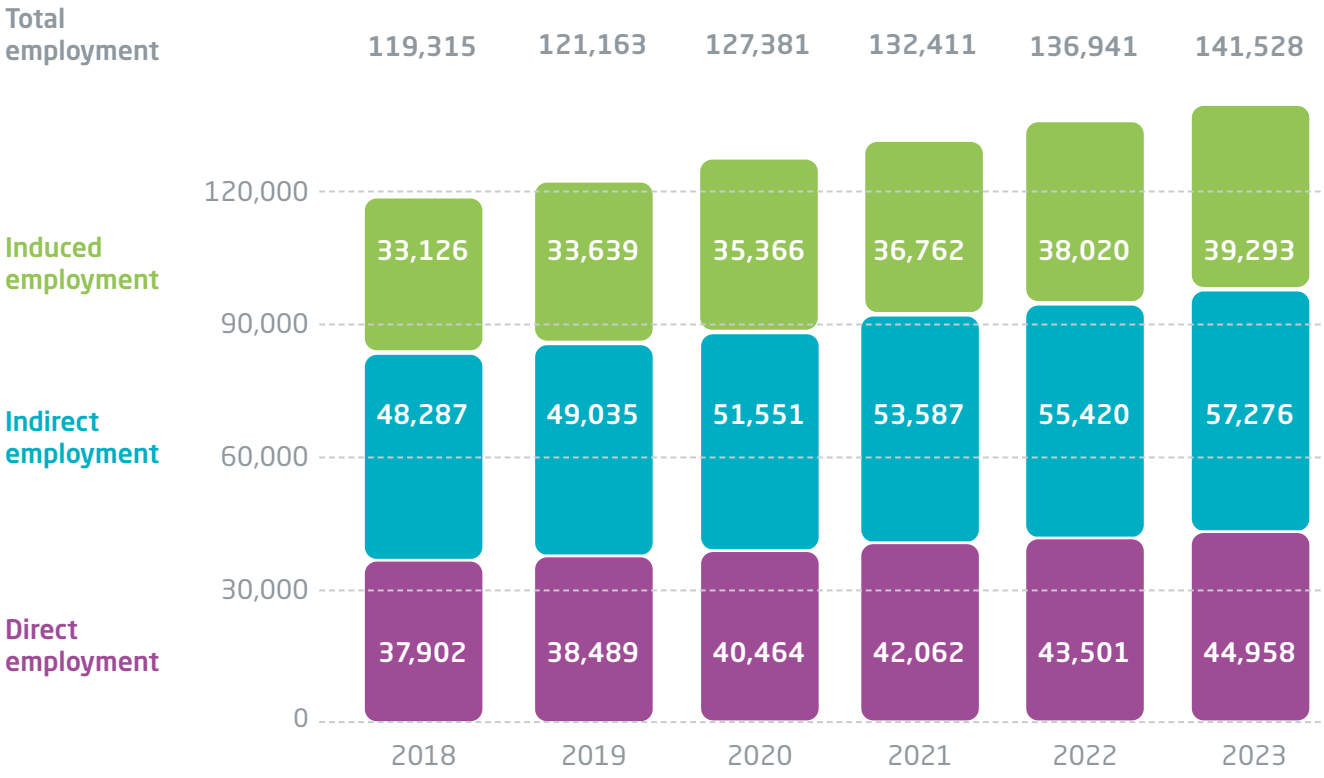
In Belgium

The biopharmaceutical sector manages to create **more jobs in Belgium time and time again**. Employment increased by about 19% in five years to 44,958 employees. Growth in jobs was significantly higher than in the manufacturing industry overall, which recorded growth of 1.7% in the same period. As a result, biopharmaceuticals accounted for some 9.3% of manufacturing jobs in 2023.

The biopharmaceutical sector's growth rates exceed those of not only the manufacturing sector but also the private sector and the Belgian economy as a whole.

However, this is only the beginning. The sector **also has a positive impact on other sectors,** such as transport and logistics. This indirect employment accounts for an additional 57,276 jobs. When including account employment generated by the spending of direct and indirect jobs, the sector accounts for 39,293 induced jobs. **This means that for every job created in the Belgian biopharmaceutical industry, two additional jobs are generated.** In total, the sector supports nearly 141,500 jobs in Belgium.

Evolution of direct, indirect, and induced employment in the Belgian biopharmaceutical sector



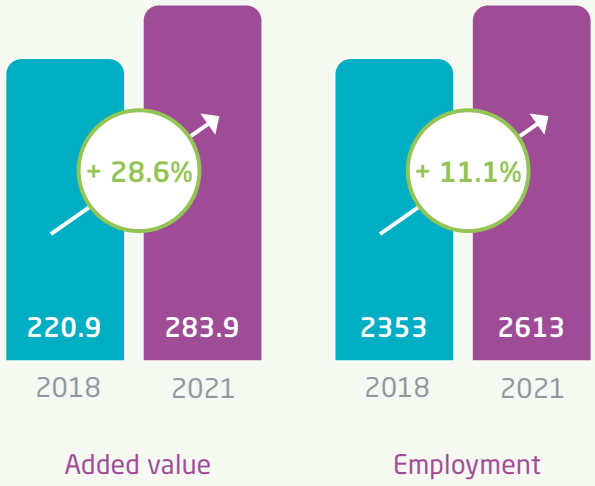
Source: pharma.be & PwC, Economic and Societal Footprint of the Pharmaceutical Industry in Europe, June 2019

Contract Research Organisations: a key player in the biopharmaceutical ecosystem

The biopharmaceutical sector has a significant positive impact on the Belgian economy, an effect that is further amplified by numerous suppliers. A prime example of these suppliers are Contract Research Organisations (CROs).

These CROs specialise in the partial or complete execution of (pre)clinical trials, making them an essential partner for biopharmaceutical companies, enabling faster market introduction of new, innovative medicines. In this way, CROs substantially contribute to Belgium's knowledge economy and strengthen the country's position as a leading centre for biopharmaceutical research and innovation.

The growing importance of the CRO Sector



Source: BeCRO, Belgian CRO Benchmarking Report, 2023

The CRO sector is thriving in Belgium. There are 135 active CROs in the country, collectively generating an added value of 283.9 million euros in 2021, marking a nearly 30% increase compared to 2018. Employment in the sector is also on the rise: in 2021, 2,613 people were employed by CROs, an 11% increase since 2018.

Internationally, the Belgian CRO sector also performs exceptionally well, with more than 20% of all European CROs active in Belgium—the highest proportion of any country.[68] Together, they generate the highest added value, accounting for about one-third of the total. In terms of employment, only Germany and the United Kingdom outperform Belgium.

The story of a remarkable partnership

Behind the numbers lies the story of a remarkable partnership between two strong entities: the biopharmaceutical sector and the CROs. Together with BeCRO, the Belgian umbrella organisation of CROs, we delve into the why, how, and what of this collaboration.[69]

Why is this collaboration so important?

"The development of medical treatments is evolving rapidly. Thanks to collaboration with CROs, biopharmaceutical companies stay at the forefront and can develop new medicines more efficiently. This has far-reaching positive impacts on both patients and society: patients gain faster access to new, more effective treatments, our body of medical knowledge expands, and healthcare system costs are reduced."

How does such a collaboration work?

"CROs and biopharmaceutical companies work in tandem, each contributing their expertise and resources to develop new, innovative treatments and deliver them to patients as quickly as possible. Biopharmaceutical companies oversee the overall strategy, specific R&D, conduct the necessary clinical trials, apply for approvals, and bear the financial risk throughout the development process. However, they don't always have the specialised knowledge and infrastructure needed to manage clinical trials."

"This is where CROs come in: their core business is setting up and monitoring clinical trials. By outsourcing this aspect, biopharmaceutical companies can focus on their core competencies. CROs offer specialised staff and state-of-the-art labs, are well-versed in regulations and the healthcare system, and maintain strong, sustainable ties with hospitals and academia. They bring efficiency and flexibility to the entire development process by adapting quickly to the specific needs of each project."

What exactly do CROs do?

"The services CROs provide are very diverse. They invest not only in highly trained staff and high-tech labs to carry out studies, but also in protocol development and oversight, patient recruitment, smooth communication between all parties involved, financial management, robust data management, safety monitoring, and ethical oversight, among other things."

In Europe

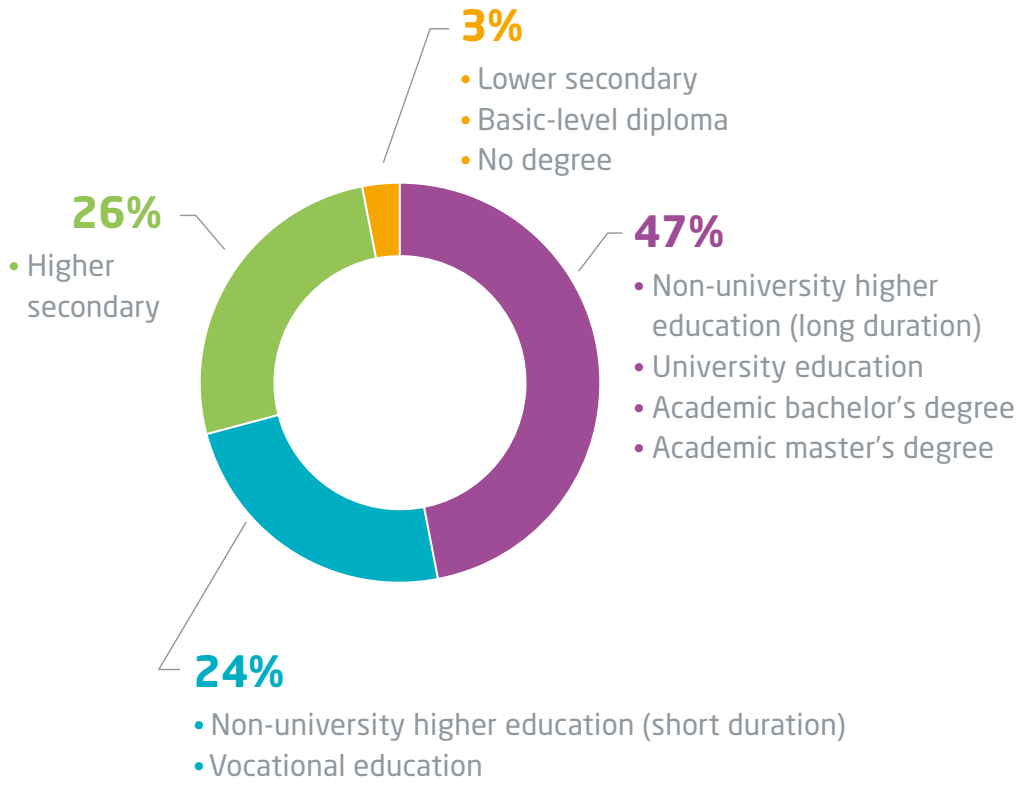
The importance of these figures becomes even clearer when you see them in a European context. Belgium ranks third regarding the share of employment in the biopharmaceutical sector (direct, indirect, and induced) as a portion of total national employment.

A diverse sector – role model for our economy

The sector not only generates a large number of jobs, but also a wide variety of jobs. The Belgian biopharmaceutical landscape is very diverse, ranging from small start-ups to highly innovative biotechnology companies and medium-sized family businesses through to local subsidiaries of multinational corporations and large manufacturing companies. This diversity is also reflected in employment. SMEs represent almost 90% of the market in numbers, accounting for about a quarter of employment within the sector.

The companies within the sector are diverse, as are the job profiles required by the industry, ranging from non-specialised to highly skilled employees. In this knowledge-intensive sector, the proportion of highly educated employees is significantly higher than in other industries, with approximately 71% holding a higher education degree. By comparison, only about half of the total working population has such qualifications. Within the broader manufacturing industry, this figure drops to 39%, while in the chemical industry, it is slightly above 50%.

Distribution of profiles in the biopharmaceutical sector in 2023



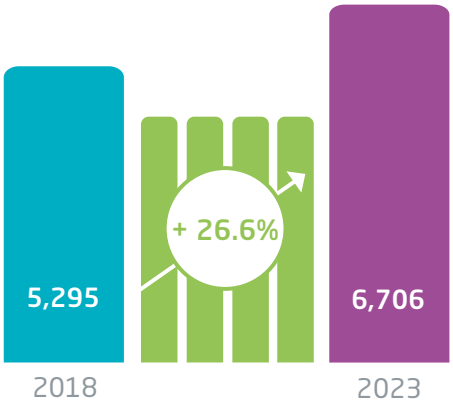
Source: Statbel labour force survey, 2023

A strong foundation

The sector has an extensive pool of skilled employees to draw from in Belgium thanks to the presence of 12 universities which provide a strong educational basis and a stable influx of highly qualified and productive employees. Some of these highly skilled staff are employed as researchers in R&D.

Over the last five years, the number of researchers working in the sector has increased by over a quarter. **In 2023, 6,706 researchers were employed in Belgium's biopharmaceutical sector,** marking an approximate 27% increase over the past five years. That is an increase of about 27% within five years. This growth rate again underlines the highly innovative nature of the sector. These employees are the cornerstone of the biopharmaceutical industry and contribute substantially to its success.

Increase in the number of researchers in 5 years



Source: pharma.be, companies that are members and conduct fundamental research in Belgium

The pharmaceutical sector has been the most attractive employer for 23 years

Each year, Randstad conducts research into the employer image of major Belgian companies and surveys the appeal of various sectors for employment. In its latest report, published in early 2024, the pharmaceutical sector emerged as the top-ranked employer. For the 23rd time in 24 editions, the sector claimed the number one spot for attractiveness,

A strong brand as an employer makes it easier for your company not only to attract people - crucial in today's war for talent - but also to retain them for longer. Employees feel more committed and are more willing to go the extra mile. However, a good reputation as an employer is not acquired overnight. It is about much more than a communication campaign; reflecting years of dedicated efforts across a broad range of areas.

The strong performance of the biopharmaceutical sector is reflected in the fact that, just like the previous year, a biopharmaceutical company tops the list again this year. Currently, the only company in the Hall of Fame – a status for companies that have achieved the first position three times in a five-year period – is also from the biopharmaceutical sector.

Attractiveness is assessed based on a combination of various criteria. A strong score in just one area is not enough for a good result; companies must perform well across all criteria to achieve a high ranking in the final list. The biopharmaceutical sector excels in this, achieving the top spot again this year, followed by the aerospace sector in second and the media sector in third place.

The pharmaceutical sector ranks highest in six criteria: work environment, job security, financial health, job content, reputation, and remuneration and benefits. These high scores match those from the previous year, showcasing the sector's consistent performance across all areas.

GLOBAL EXPORT

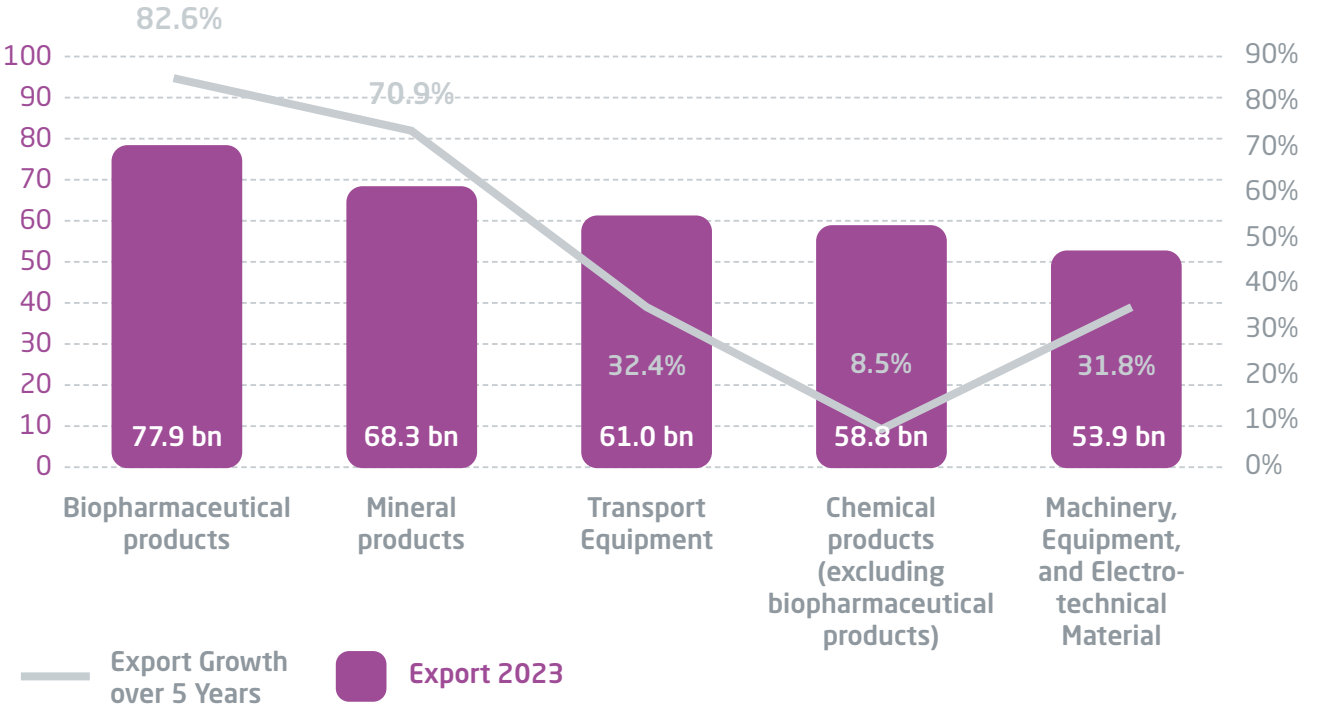
In Belgium

During the COVID-19 pandemic in 2021 and 2022, the Belgian biopharmaceutical sector played a crucial role in manufacturing vaccines. The unprecedented scale of that effort is also evident in its export figures: in 2022, exports of medicines and vaccines reached almost 100 billion euros. This confirms Belgium's unique position as an international hub for medicines.

In 2023, COVID-19 vaccine exports fell sharply due to the end of the pandemic. As a result, the sector's total exports fell by about 20%, to 78 billion euros. However, over a five-year period, exports continued to grow by more than 80%. When looking at the numbers excluding COVID-19 vaccines, exports in the sector have continued to grow by more than 55% over the past five years.

With a 14.8% share of total exports, the biopharmaceutical sector remains Belgium's export champion. Biopharmaceutical exports have grown faster than any of the other top five sectors' exports over the past five years. The biopharmaceutical sector is thus taking an increasing lead and is gaining importance within the export-oriented Belgian economy.

Top five Belgian export sectors

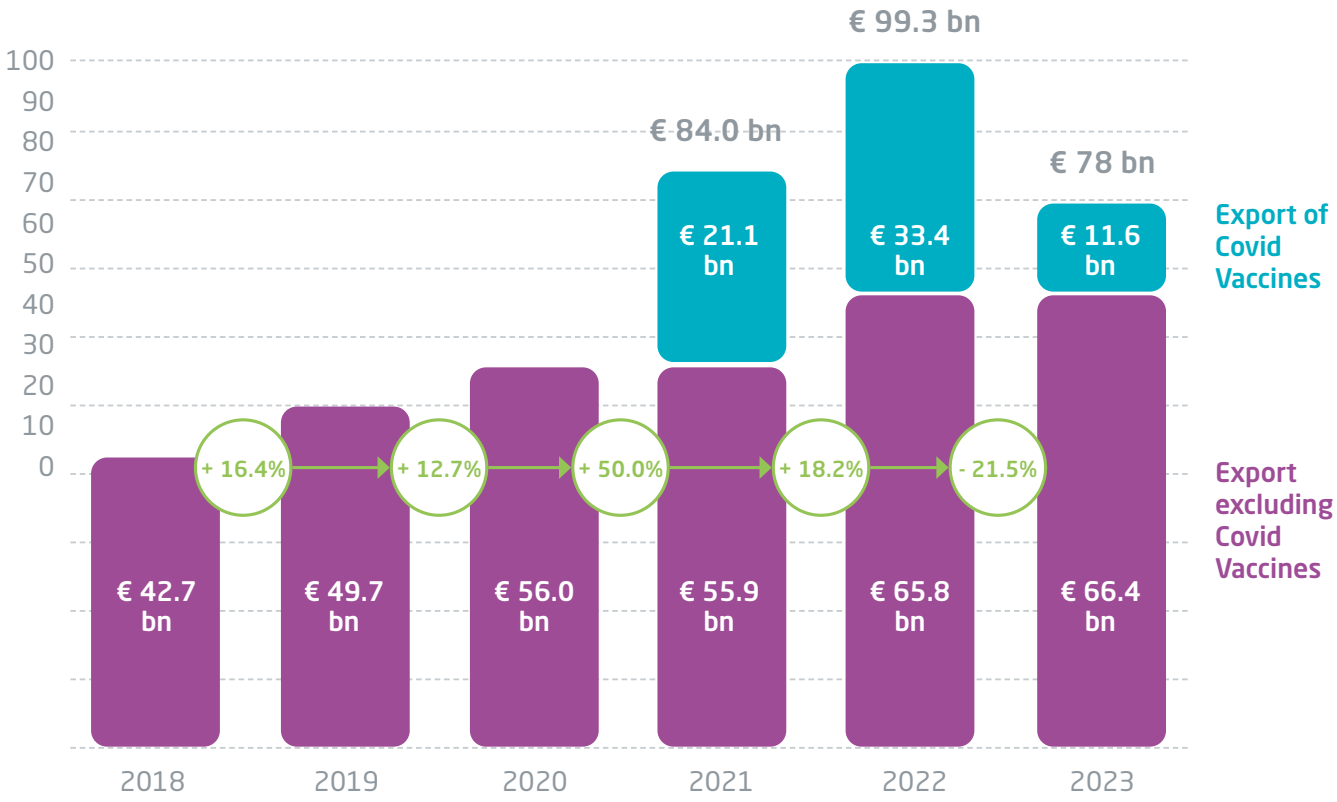


Source: NBB

Every day, over 213 million euros worth of biopharmaceuticals are exported from Belgium. This resulted in a trade surplus of 9.2 billion euros in 2023. Belgium's overall trade balance shows a surplus of 16.3 billion euros.

The biopharmaceutical sector therefore accounts for 56% of Belgium's trade surplus, by far the largest share of any sector.

Evolution of total exports in the Belgian biopharmaceutical sector



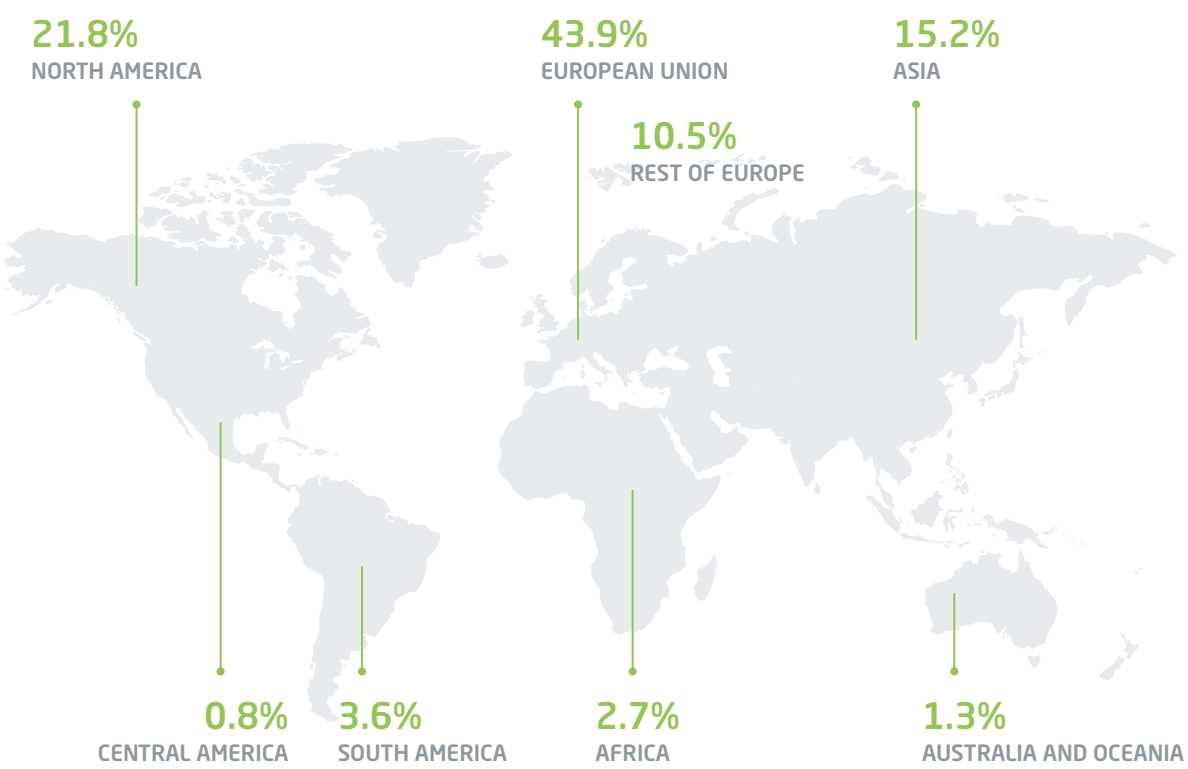
Source: NBB

Comparison with Europe and the world

From a global perspective, Belgium is performing exceptionally well. Within the EU, Belgium ranks second in total biopharmaceutical exports, with only Germany ahead. Belgium ranks third per capita, after Ireland and Slovenia. **In total, almost 15% of total EU biopharmaceutical exports are shipped from Belgium.**

More than half of biopharmaceutical sector exports leave the European Union. In total Belgian exports, this is less than a third. The United States is by far Belgium's main trade partner, representing nearly 20% of Belgium's biopharmaceutical exports. This is followed by Germany and Italy, with 11.9% and 7.7% respectively.

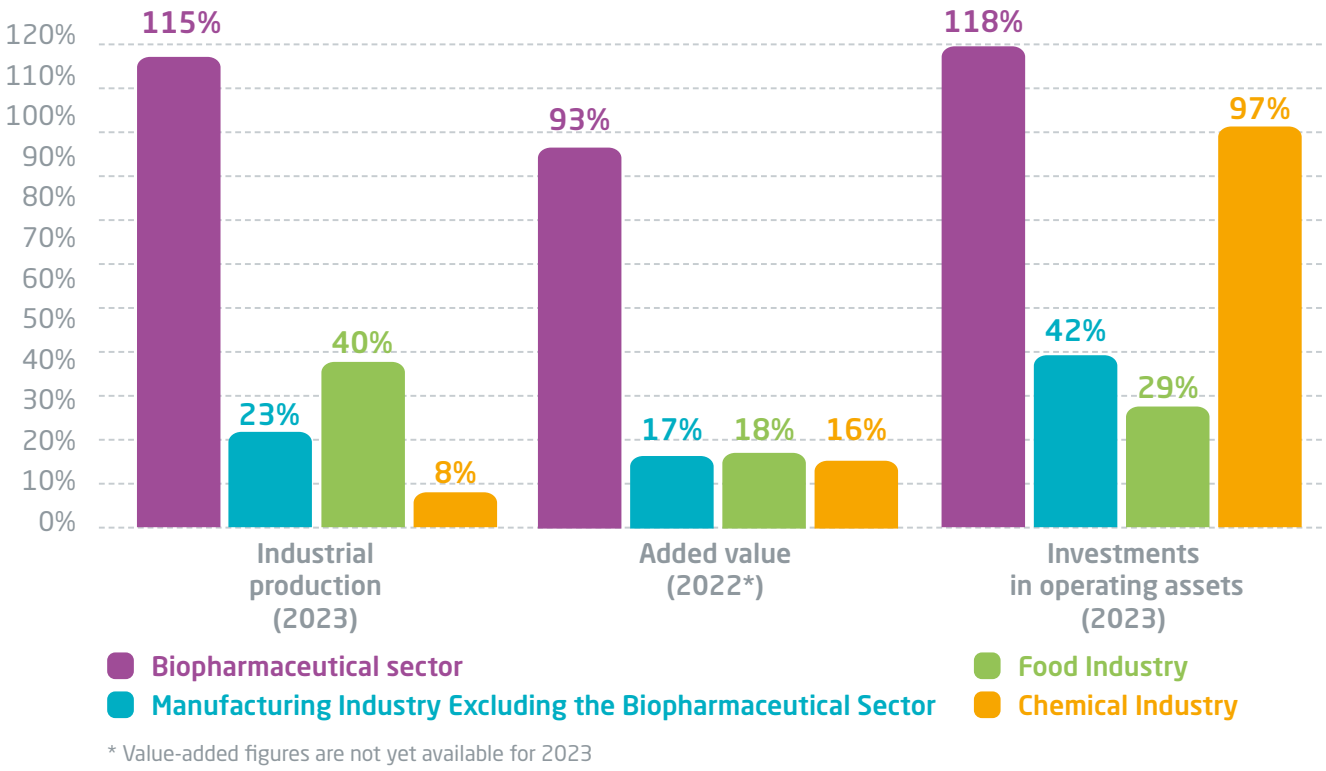
Exports in Europe... and far beyond



Source: NBB

PRODUCTION, ADDED VALUE AND INVESTMENTS

Evolution of added value, investment and production over five years



* Value-added figures are not yet available for 2023

Source: NBB, Value Added (at Basic Prices), Statbel, Industrial Production Index, Workday-Adjusted Index, Statbel, Turnover and Investments based on VAT Declarations

Production

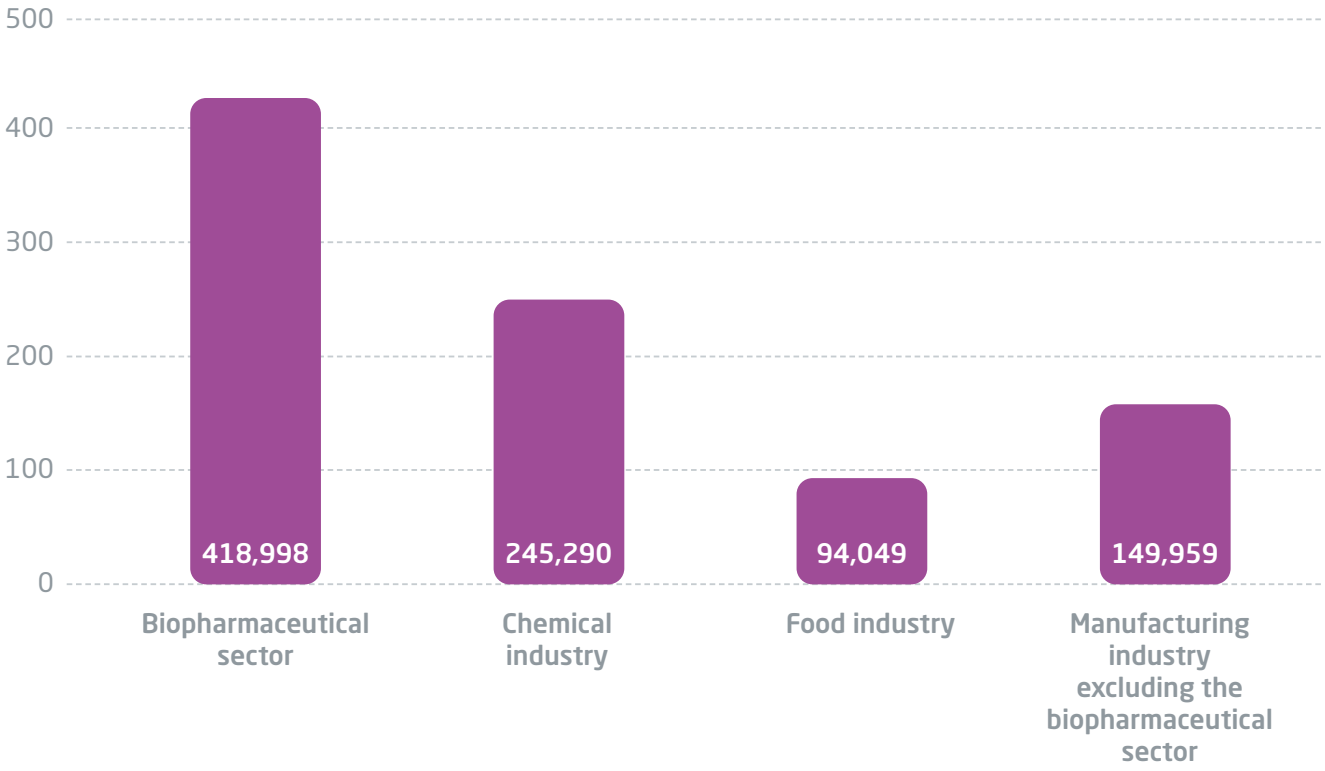
In 2023, total industrial output in Belgium's manufacturing sector declined, including in the biopharmaceutical sector, primarily due to a sharp drop in COVID-19 vaccine production. However, looking over the past five years, biopharmaceutical production has more than doubled – a growth unmatched by any other industrial sector.

Added value

In terms of added value, the biopharmaceutical sector is also performing exceptionally well. In just five years, the added value has doubled, making biopharmaceuticals the leading sector in terms of added value since 2019. No other sector can boast higher growth rates. **By 2022, the biopharmaceutical sector accounted for almost a fifth of the total added value generated by the manufacturing industry in our country.** Over the past 25 years, the added value of the biopharmaceutical sector has quadrupled.

This impressive performance is largely driven by the sector's high employee productivity. With a gross added value of nearly €420,000 per employee, it is almost three times higher than the average in other manufacturing industries. Internationally, this is an outstanding achievement, ranking Belgian biopharmaceutical employees second within the EU27. This achievement clearly underscores the importance of the sector to the economy, as Belgium's outcome is more than double the EU27 average.

Added value per employee (2022)



Investments in operating assets

The sector's impressive performance is partly due to the tremendous efforts in R&D and continuous investment in operating assets. The investments encompass land, buildings, installations, machinery, and equipment, aimed at expanding production capacity or enhancing the environmental sustainability of production facilities. Over five years, investments in operating assets have more than doubled, with the sector accounting for almost 10% of manufacturing investment by 2023. No other sector can boast such growth.

3.2.2 Cost-benefit analysis for the Belgian government

The direct economic impact of Belgium's robust biopharmaceutical sector is evident. Additionally, the sector positively influences public finances, as demonstrated by the following calculation based on 2023 statistics.

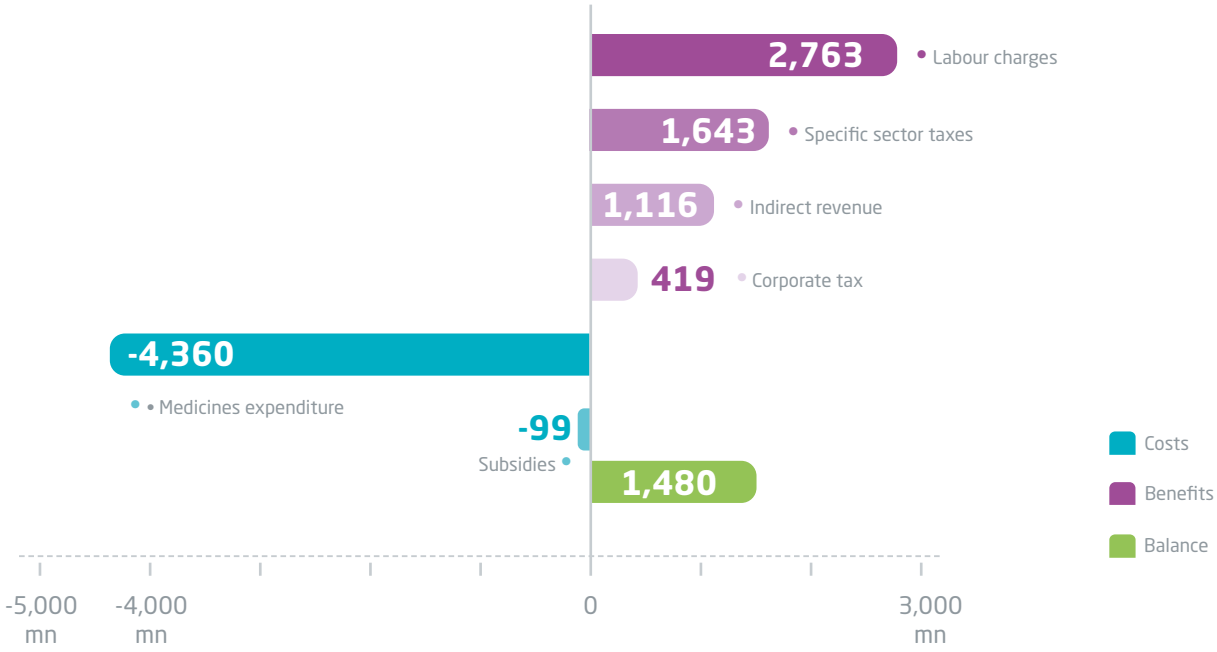
To create this cost-benefit analysis (see also Appendix 1), we first consider the costs incurred by the government for medicines. These costs are reimbursed by the NIHDI and include expenditures on all reimbursed medicines. Government subsidies for the biopharmaceutical industry are also included here. In total, these expenditures amount to 4.5 billion euros.

The biopharmaceutical industry, however, also generates significant revenues for the government, including:

1. Taxes on labour, amounting to almost 2.8 billion euros
2. Sector-specific levies (such as turnover tax)
3. Corporate taxes
4. Revenues linked to the economic chain created by the biopharmaceutical sector

In total, government revenues amount to €5.9 billion.

Costs and benefits of the biopharmaceutical sector for Government Finances



This comparison of government expenditure and revenue highlights that the biopharmaceutical sector's contribution to Belgium's revenue significantly exceeds the government's spending on the sector.

The surplus amounts to almost 1.5 billion euros. A comparison with other countries shows that this position is unique, and the envy of many countries.

Our approach 4

4.1 We take responsibility

4.1.1 Following strict ethical standards

The biopharmaceutical sector is rightly one of the most regulated in Belgium. It plays a crucial role in ensuring an invaluable asset: our health and quality of life. pharma.be fully embraces this responsibility. Since its founding, pharma.be members have adhered to strict ethical principles that go beyond the legal minimum standards. **Care, integrity, respect, and honesty** serve as guiding principles.

pharma.be members uphold these core values not only within their own operations but especially in their interactions with others. **Collaboration with knowledge institutions, governments, healthcare professionals, patients, and patient organisations is essential.** Effective, innovative treatments cannot be developed without these interactions. **These core values ensure that the patient's interest always remains central, and that every collaboration is transparent, independent, and of high quality.**

These core values are also formally guaranteed. pharma.be first introduced its own Code of Deontology nearly 50 years ago, making it the first Belgian industry federation to do so. The core values have remained unchanged, but their translation in the Code of Deontology has evolved to address new ethical challenges arising from rapid advancements in science, technology, and society. In 2024, pharma.be revised its Code of Deontology to further support increased collaboration with patients and patient organisations.

pharma.be upholds its core values not only through the Code of Deontology but also through the **Bureau van Toezicht op de Geschreven Communicatie (BTGC, Bureau for Control on Written Communication)**, an independent body launched in 2010 to enhance the quality of communication with healthcare providers. These two initiatives highlight pharma.be and its members' dedication to transparency, accuracy, and high-quality work for the benefit of patients.

However, fair and quality care extends beyond the scope of pharma.be's operations. In addition to its own initiatives, pharma.be actively contributes to broader collaborations and strategic tools. pharma.be is part of the **Mdeon ethical platform and Betransparent.** As a representative of the innovative biopharmaceutical sector, pharma.be strives to ensure that these initiatives evolve in line with the changing context and patient needs.

Code of Deontology updated in dialogue with patients and patient organisations

For nearly 50 years, pharma.be members have been committed to their own Code of Deontology, providing a framework for building sustainable relationships with all healthcare partners, from academic institutions and doctors to patient organisations. In recent years, pharma.be's dialogue with patients and patient organisations has intensified, as reflected in the roundtable discussions held on 28 November 2023. During this dialogue, patients and patient organizations expressed that the complaint procedure outlined in the Code of Deontology is perceived as burdensome and overly formal.

In response to this feedback, pharma.be **adjusted the Code of Deontology in 2024 to better accommodate the needs of patients and their organisations.** Until now, individuals or organisations with concerns about a pharma.be member's compliance with the Code could **file a complaint** with the **Secretariat of the Code of Deontology**. This **complaint** was **reviewed** by the **Committee for Ethics and Pharmaceutical Deontology (DEF Committee)**.

The recent amendment to the Code, approved by the General Assembly on 13 March 2024, now allows for **informal exchange and mediation** between a patient or patient organisation and a pharma.be member.

With growing collaboration between patient organisations and innovative biopharmaceutical companies, it is crucial to ensure a level playing field in case of disputes. The addition to the Code of Deontology is a testament to this valued partnership and a sign of mutual trust.

Stefan Joris and Inge Van de Velde, members of the Patient Advisory Board of pharma.be

Additional role for the BTGC

Patients or patient organisations wishing to use this new option can submit a complaint form online or by post. The chair of the BTGC (see further) verifies the admissibility and conducts an initial review of the complaint's validity. **If the complaint meets both criteria, the parties involved are invited to an informal meeting led by the BTGC chair.** This meeting allows the parties to speak openly without lawyers or legal representatives.

Through this process, the parties can **exchange viewpoints and reach reconciliation** in an accessible manner. Reconciliation is verbal and non-binding. No fees are charged for this informal procedure. This procedure applies only to potential violations of the Code of Deontology; complaints regarding adverse effects or other product issues are beyond its scope.

DEF procedure also more accessible if no agreement is reached

If the reconciliation meeting does not result in an agreement, the parties may still initiate a **formal procedure with the DEF Committee**. In such cases, the revised Code of Deontology ensures that the DEF procedure, like the reconciliation process, remains free of charge for the patient and/or patient organisation. Additionally, they may choose the language of the procedure – Dutch or French – without being restricted by the language of the pharma.be member's registered office location.

Through this amendment to the Code of Deontology, pharma.be aims to foster an ethical, transparent, and sustainable partnership with patients and patient organisations.

Core task of the BTGC: verification of written communication

The BTGC has expanded its role to include managing the informal complaints procedure related to the Code of Deontology. However, this does not affect its core responsibility: **overseeing and supporting member companies in producing fair and high-quality written communication. The BTGC focuses specifically on communications about medicines directed at healthcare professionals** and does not extend to communications exclusively aimed at patients.

Why?

In Belgium, advertising for medicines for human use is strictly regulated by the government. This legislation aims to promote rational medicine use, by ensuring objectivity and providing accurate, complete information.

To **assist member companies**, the BTGC – an independent body composed of a lawyer, physician, and pharmacist – was established in 2010. They ensure that company communications comply with the Code of Deontology and all relevant laws and regulations. They also provide recommendations for enhancing the quality of communications. This self-regulation system is a world first.

Oversight Body

The BTGC annually reviews communications for fifty medicines from fifty different pharma.be member companies. The companies provide all written communication for the selected medicine to the BTGC. The BTGC checks for compliance with the pharma.be Code of Deontology, adherence to legal requirements, inclusion of essential details, and clarity and accuracy of information. Each company receives a report with preliminary findings. After receiving feedback and/or additional information, the BTGC prepares a final report, enabling the companies to adjust their communications accordingly.

Advisory Role

The BTGC is not only an oversight body but **also an advisor and sounding board**. It closely monitors laws and regulations while staying attuned to communication trends, including online and cross-media approaches. With its reports, the BTGC possesses **a wealth of information and expertise**. From these, it highlights best practices and provides concrete recommendations. These insights are summarised in the BTGC's annual report.

Through this oversight process, the BTGC ensures that our companies remain vigilant regarding the Code of Deontology and applicable laws and regulations, adhering strictly to them. Additionally, practical examples and recommendations help maintain the integrity and quality of written communication amidst all trends and developments.

BTGC Annual Report 2023: Figures, Conclusions & Tips

Figures

- The BTGC analysed 50 medicines from 50 different members.
- In 68% of cases, members provided comments and/or additions to the preliminary report,
- And 50 final reports were issued.

General Conclusion

- In 2023, pharma.be members generally adhered well to ethical and regulatory requirements for written communication.
- The fact that 68% responded to the preliminary reports demonstrates that companies prioritise clear and high-quality communication. Most responses either confirmed the implementation of necessary adjustments or offered further clarification.

Tips

- A broader range of communication channels is increasingly used, requiring extra attention to keep information accurate and easily accessible across platforms. For instance, companies may not always repeat prices or other essential elements in online communications but instead include a link. While this approach simplifies updating information, it should not make finding information difficult for readers. The BTGC requests that links direct readers straight to the necessary details without multiple clicks.
- Graphical representations are frequently used as well. These can convey information more quickly, but like written text, they must be clear and comprehensive. The BTGC advises providing additional explanations for graphs and similar visuals where necessary to avoid confusion or misinformation.
- The use of technical terms or specific abbreviations also poses a risk of confusion or misinformation. While these terms may be familiar within biopharmaceutical companies, readers may not always recognise them or might interpret them differently. The BTGC recommends clarifying specific terms and abbreviations.

Mdeon: Ethical Health Platform

Mdeon consists of around thirty associations, including physicians, pharmacists, nurses, paramedics, hospitals, and the biopharmaceutical industry. Under Article 10 of the Medicines Act, **Mdeon** aims to provide a **high-quality framework for information and promotion of medicines and medical devices**.

For instance, to properly inform healthcare professionals about the latest developments, biopharmaceutical and medical technology companies frequently organise scientific events. In some cases, a visa is required to finance healthcare professionals' participation in such events. Mdeon grants these visas, ensuring compliance with applicable legislation and ethical standards.

Each year, Mdeon publishes a detailed activity report on the visa process. The 2023 key figures can be found here:

Key figures for 2023

Betransparent: transparency in the interest of patients

For biopharmaceutical companies, partnerships with healthcare professionals, healthcare organisations, patients, and patient organisations are crucial. These collaborations range from **sharing expertise** and participating in scientific research **to sponsoring medical education**. **Only through close collaboration can the biopharmaceutical sector continue to foster innovations and breakthroughs that significantly improve patients' lives and quality of life.**

Equally crucial is ensuring the independence of the partners the sector works with. This requires not only **strict laws and regulations** but also **transparent reporting**. Open and clear communication about these interactions fosters better understanding and increased trust in the sector. pharma.be has long emphasised transparency in its Code of Deontology. Since the Sunshine Act of 2016, transparency about (direct and indirect) fees and benefits provided by the sector to partners has also been legally anchored in Belgium.

Following the Sunshine Act, anyone can find financial information on www.betransparent.be regarding the sector's interactions with healthcare professionals, healthcare organisations, and patient organisations. This includes fees for services and consultancy, support for organising or participating in scientific events, and grants to support healthcare. The transparency register is published annually. In 2023, pharma.be contributed to making this register easier and more widely searchable.

Code for Betransparent.be

Transparency Register 2023: figures and trends

Figures

- A total of 688 biopharmaceutical and medical technology companies published their data for 2023.
- They invested a total of €303,425,820 in collaborations with healthcare providers, healthcare institutions, and patient associations active in Belgium.
- This investment is distributed across various types of collaborations, as illustrated below:

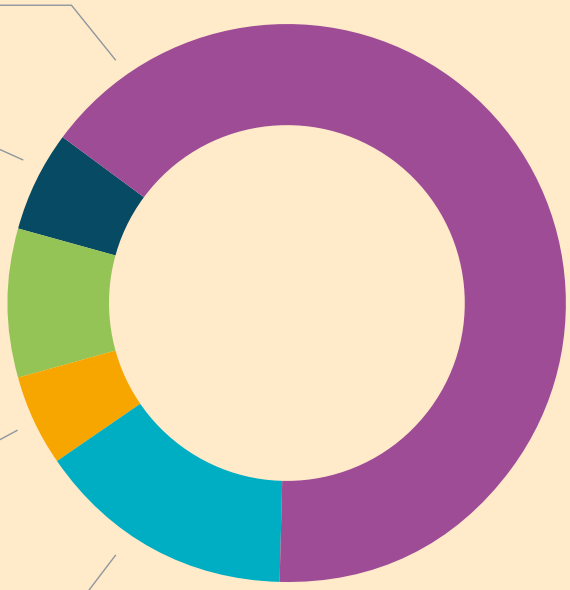
€ 198.5 mn
Scientific research

€ 17.3 mn
Support for patient organisations

€ 26.2 mn
Consultancy services

€ 16.0 mn
Donations and grants

€ 45.4 mn
Scientific events



Trends

Looking at the figures from the past five years, we observe a general increase in collaboration between the industry and the healthcare sector, except for a notable decrease during COVID year 2020.

Investments in 2023 were 13% higher than the previous year and 22% higher than in 2019. However, the growth varies across different types of collaborations. For instance, investments in scientific research have risen by nearly 30% compared to five years ago, while donations and grants have declined by 27%.

A key trend is the intensified collaboration with patient organisations. Although investments in this area accounted for only 6% of the total in 2023, this still represented an approximate doubling compared to 2019. This shift highlights the growing importance placed on incorporating the patient's perspective throughout the biopharmaceutical innovation journey.

4.1.2 Addressing urgent patient needs

The compassionate use and medical need programs allow, in exceptional cases, the administration of a medicine still under review and not yet approved by the EMA, to patients with chronic or serious diseases who have no viable treatment options with existing market-approved medicines.

In 2014, Belgium adopted European Regulation 726/2004 (Article 83) through new legislation, introducing the concept of compassionate use. Simultaneously, the country expanded the legal framework by also establishing Medical Need Programmes.

The main difference between these two programmes lies in their relationship to marketing authorisation:

- *Compassionate use programmes* (CUP) apply to medicines that have not yet received marketing authorisation.
- Medical Need Programmes (MNP) involve medicines that already have a marketing authorisation for a specific indication but are being considered for use in an unapproved indication.

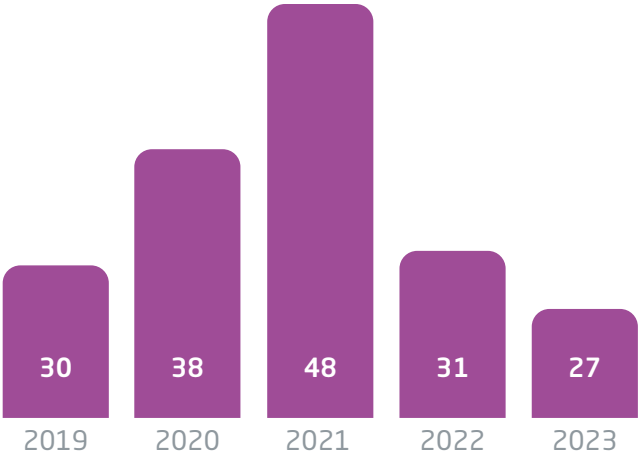
To give patients **faster and free** access to the latest treatments through these programmes, in exceptional cases, even before the registration procedure is completed, the FAMHP needs to grant a temporary authorisation or Early Temporary Authorisation (ETA).

Because the EMA has not yet granted market authorisation, the FAMHP has to strictly weigh the benefits versus the risks of the medicine. If the application is approved, the company gives the new medicine free of charge to patients who are included in the programme at the request of their treating doctor. The programmes remain in effect until the medicine becomes available on the market for the specific indication in question.

This exceptional provision is made in full transparency; all approved programmes are published on the FAMHP website.[70] Patients and healthcare providers can consult them there.

By the end of August 2024, more than 42 programmes were ongoing.

Number of compassionate use and medical need programmes submitted to FAMHP over the last five years



Decisive measures and collaboration needed

Despite advancements, approximately 95% of identified rare diseases still lack an approved treatment.[72] To address the challenges in the search for new treatments, decisive policy measures and public-private partnerships are essential.

A prime example of such a partnership is the **Rare Disease Moonshot initiative** – a coalition of eight international partners from industry, government, science, and patient communities. Launched at the end of 2022, this coalition aims to accelerate breakthroughs in therapies for rare diseases and currently untreatable childhood diseases.

The Belgian government also aims to expedite progress. On Rare Disease Day in 2024 (held annually on the last day of February), the Federal Minister of Health announced the development of a new Belgian plan for rare diseases. In this context, pharma.be also advocates for cooperation and synergy between the government, industry, and patient organisations.

This was also highlighted at the Rare Diseases Forum held on 16 April 2024 during Belgium's presidency of the Council of the European Union. As a co-organiser, pharma.be, along with nine other European partners, brought together patient organisations, companies, and governments to discuss how the European competitiveness in R&D for rare diseases can be maintained. Panel member Caroline Ven, CEO of pharma.be, highlighted the need for adequate budgets for reimbursing innovative treatments, including orphan medicines.

4.1.3 Through education and dialogue

In 2023, pharma.be launched an **educational information campaign** aimed at various audiences within the healthcare ecosystem. Many topics related to health and medicines, in particular, are complex, and pharma.be seeks to provide clearer answers to pressing questions. Information on why certain problems can only be solved through cooperation and, most importantly, what the sector is doing to improve health for everyone in Belgium. Following a successful first edition, a second wave was launched on 7 October 2024, with a focus on the value and availability of medicines.

This year, pharma.be once again engaged with numerous stakeholders across Belgium. As part of the "**pharma.be on tour**", (future) policymakers and public stakeholders were invited to visit member companies for a series of interesting debates. By listening to each other and not shying away from critical discussion, we can make progress.

Media publications, some of which were highly critical of the pharmaceutical industry, presented a challenge in engaging with journalists, offering context, and clarifying the complexities of existing systems and regulations.

The **pharma.be** team also participated in various **panel discussions** and **debate programmes** on topics such as the **(temporary) unavailability of medicines, transparency in government conventions, and the value of medicines**.

Below, we explore three key themes around which pharma.be has been actively engaged in dialogue over the past year.

A future-proof and patient-centred reimbursement procedure for medicines

Belgium's **current reimbursement procedure** for medicines, unchanged since 2001, **no longer meets the needs** of today's **rapidly evolving scientific and technological innovations**.

To address this, the NIHDI began an extensive consultation in 2022 with stakeholders such as the government, patient organisations, the healthcare sector, and the biopharmaceutical industry, including pharma.be. The need for collaboration among different stakeholders is aimed at ensuring that the reforms meet the needs of patients and healthcare providers. pharma.be played an active role in these discussions.

The future-proof system should be innovative and responsive, with transparent and inclusive decision-making.

This led to the development of a "**roadmap**" **for reforming the reimbursement process, which will be** implemented in stages starting on 1 January 2025, with full implementation within two years.

Key components of the new reimbursement system advocated for by pharma.be:

- A major pillar of the new system is the strengthened involvement of patient organisations in the decision-making process regarding medicine reimbursement. The Commission of Reimbursement of Medicines (CRM) will be expanded to include representatives who amplify the patient's voice, contributing to more patient-centred decisions.
- For the first time, patients and patient organisations are directly involved in reimbursement decisions for medicines.
- Procedures are streamlined and accelerated, providing patients with faster access to new and innovative medicines, which is literally life-saving for patients with severe or rare conditions with limited or no other treatment options.
- Reimbursement becomes more transparent, giving patients, patient organisations, and healthcare providers quicker, more comprehensive, and clearer information on medicine availability and reimbursement.

pharma.be actively supports the rollout of these essential reforms, aiming to bring innovations from the biopharmaceutical sector to patients in a fast and affordable manner within the healthcare system.

"Stronger than cancer together!"

Since 2019, cancer has been the leading cause of death in Belgium, claiming approximately 228 lives daily. The current cancer plan has delivered significant results but dates back to 2008. Needless to say, much has changed since then. On one hand, there have been tremendous advancements in technologies, innovative therapies, and insights, while on the other the challenges and needs of patients and healthcare providers have evolved. Despite the annual rise in cancer diagnoses, more people are now surviving after their diagnosis.

In 2022, the Belgian Foundation against Cancer, Belgian Board of Oncology, Belgian Cancer Registry, and Sciensano published the Belgian Cancer Barometer, highlighting improvements needed in areas such as prevention, screening, diagnosis, treatment, aftercare, life after cancer, and palliative care.

Thanks to the Belgian mirror group for Europe's Beating Cancer Plan (EBCP), Belgium is actively involved in numerous EU-funded projects; however, there remains a need for a Belgian vision with a more integrated approach, clear objectives, and accompanying funding.

Currently, Sciensano's Belgian Cancer Centre is developing a new framework, the Belgian Cancer Inventory, to monitor cancer care and policies in Belgium.

On 14 March 2024, pharma.be's Cancer Plan focus group, together with the Belgian Society of Medical Oncology (BSMO), the Belgian Haematology Society (BHS), and the Belgian Society of Paediatric Haematology Oncology (BSPHO), organised a roundtable. Participants included representatives from the Belgian Foundation against Cancer, Kom op tegen Kanker, Belgian Cancer Registry, FOD Public Health, Sciensano Cancer Centre, the Belgian Board of Oncology, policymakers, the Patient Expert Center, and the patient organisation Prolong.

A strong partnership between the government, healthcare professionals, pharmaceutical companies, and patient organisations is essential for improving the quality of life for cancer patients and their families.

Elke Stienissen, Chair of the Patient Committee of the Belgian Haematology Society

Reimbursement of cancer medicines for children

Starting **1 January 2024,** the NIHDI launched a **reimbursement project for cancer medicines for children.**

Pediatric oncologists frequently prescribe off-label medications for children because of the limited availability of cancer treatments specifically developed for them. Although these medicines were initially developed for adults, they are also clinically effective for children. However, off-label medicines were not always reimbursed, often leaving parents with high costs.

This project addresses that issue. pharma.be supports this approach to temporary access, as it is based on the available scientific evidence for administration to children under the supervision of the NIHDI monitoring committee.

This solution ensures that standard treatments for children with cancer can be continuously adapted and improved, providing the best possible support for children with cancer and their families.

Children with cancer represent a very specific group and therefore require a unique focus.

Prof. Dr. An Van Damme, Belgian Society for Pediatric Hematology and Oncology

4.1.4 Caring for the environment and climate

Two years of the Green Deal Duurzame Zorg (Sustainable Care)

In 2023, pharma.be contributed to the establishment of the **Green Deal Duurzame Zorg**. This **initiative** by the **Flemish government** aims not only to prepare our **healthcare system for the effects of climate change and pollution**, but also to unite **people and organisations**. It fosters collaboration and idea-sharing to promote sustainability through concrete actions.

Four main themes were identified within the Green Deal: 'Nature & Health,' 'Climate & Infrastructure,' 'Materials & Waste,' and 'Medicines in Water.' pharma.be actively participates in the last two themes and co-leads the 'Medicines in Water' initiative alongside Vlakwa (Flemish Water Knowledge Centre) and the Flemish Environment Agency (VMM).

Within **'Materials & Waste,'** we collaborate with others to raise awareness of sector measures for reducing waste and **properly collecting expired and unused medicines** (see next paragraph).

For **'Medicines in Water,'** after an exploratory process, specific actions were proposed, aiming to achieve concrete results by the end of the Green Deal in 2026.

This includes increased collaboration with surrounding regions, identifying priority medicines from the European monitoring list in Flanders, and **raising healthcare professionals' awareness** of the **potential environmental impacts of medicines, especially on the water ecosystem**.

Through these concrete actions, we work with doctors, pharmacists, and hospitals towards a healthy living environment where environmental care and the right treatment for humans and animals go hand in hand.

Multistakeholder collection of expired and unused medicines

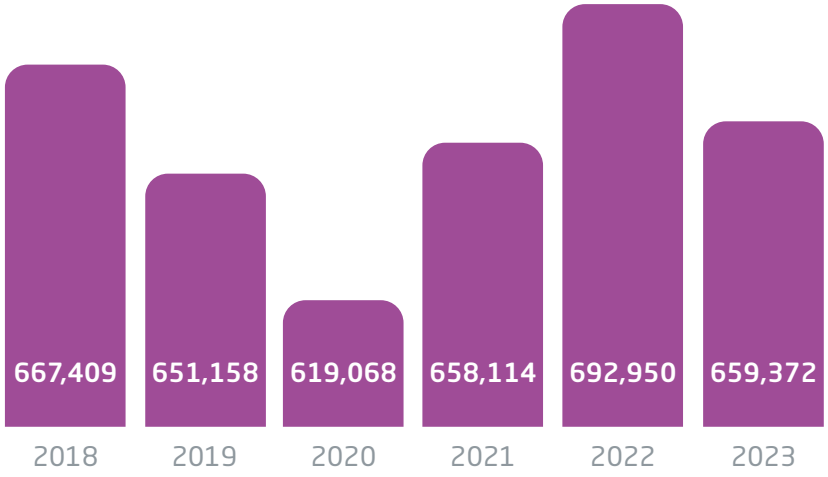
Another example of pharma.be members' efforts to reduce their environmental impact is the **collection of expired and unused medicines.** You can't just flush those down the toilet or dispose of them in the rubbish bin. They must be sorted and collected separately, as they can be harmful to the environment and to public health. After all, unused medicines should not be used or disposed of carelessly (children playing could find them, for example, or animals looking for food).

To encourage sorting and ensure proper collection, the biopharmaceutical sector has collaborated with various stakeholders to develop a convenient zero-cost solution to the patient. Thanks to this **multistakeholder initiative**, you can simply drop off unused and expired medicines to your pharmacy. The pharmacist collects the medicines in a special cardboard box. The boxes are collected by wholesale distributors and incinerated; the thermal energy that is released during the process is reused.

The biopharmaceutical industry bears the costs of the cardboard boxes and incineration, including transport costs from the wholesaler's distribution centre to the incinerator. These costs are allocated based on the number of medicines sold on the outpatient market in the previous year.

Thanks to this collaboration, an estimated 659,372 kg of unused and expired medicines were collected in Belgium in 2023.

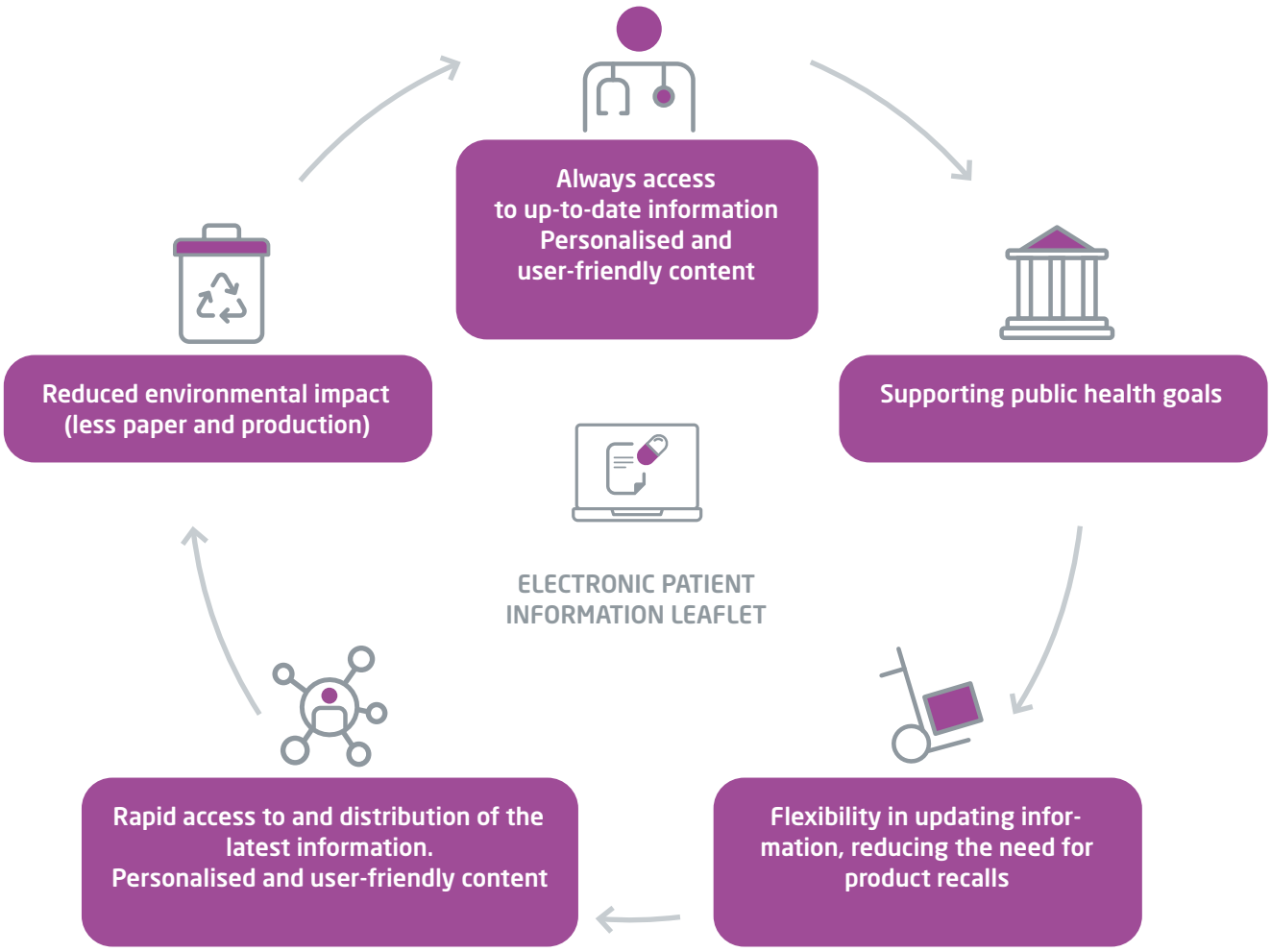
Total amount of collected expired or unused medicines in kg



Source: pharma.be

From paper to electronic medicine information

Currently, EU regulations still require a paper leaflet with necessary information and instructions for the safe and effective use of each medicine. In Belgium alone, more than 100 million packs are dispensed each year for reimbursed medicines. Paper leaflets have a significant environmental impact.



e-Pil, a European first

To address this, Belgium's biopharmaceutical industry began testing an electronic leaflet, the **Electronic Patient Information Leaflet** (e-Pil), in 2018 – a European first. Initially, the e-Pil was used only for a limited number of medicines in a hospital setting. It was made accessible through reliable sources like the FAMHP database, the BCFI (Belgian Centre for Pharmacotherapeutic Information) website, and pharma.be's e-compendium website.

The interim evaluation in 2022 was particularly positive, not only regarding environmental impact but also in terms of ease of use: thanks to the e-Pil, users always have access to the most up-to-date information, which is easier to read and search. The e-Pil certainly does not fall short of the paper leaflet – quite the opposite. As a result, from 2023, the pilot project was expanded to a broader selection of medicines.

Europe also seems to be in favour of the e-Pil

The positive effects have not gone unnoticed in Europe. As part of the ongoing revision of pharmaceutical legislation, the European Commission aims to enable the transition from paper to electronic leaflets. Member states would individually determine the (gradual) rollout. The Commission emphasises, however, that patients should always retain the right to request a paper leaflet.

The European Parliament expressed its opinion on this proposal from the European Commission in April 2024, noting that it is up to member states to decide whether the leaflet should be provided electronically only, or both electronically and on paper. Such a decision, according to the European Parliament, should be made based on input from patients, healthcare providers, and other stakeholders. The European Council is expected to review the proposal at the end of 2024.

A new test case

The decision-making process at the European level will likely not be finalised until 2025, but in the meantime, the Belgian biopharmaceutical industry is keen to continue preparing for the transition. A starting point for this preparation is the final evaluation of the Belgian pilot project in October 2024. pharma.be is currently exploring the possibility of setting up a new test case, this time outside the hospital environment. This will allow the industry to optimally prepare for the transition from paper to electronic leaflets.

In designing this new test case, pharma.be is guided by the following key questions:

- What do we aim to achieve concretely with the test case?
- Which aspects do we want to measure, and how will we approach this?
- What type of medicines would be most suitable to include?
- How can patients and healthcare providers best access the electronic leaflet?

To obtain well-founded answers, pharma.be is engaging in dialogue with all stakeholders. Open collaboration between the industry, governments and administrations, healthcare providers, and patients is essential to ensure a smooth transition to electronic leaflets, with respect for the needs of all parties involved.

From paper to electronic patient information

ESG and the biopharmaceutical sector

What does ESG stand for?

ESG stands for **Environmental, Social** and **Governance.** It refers to three key factors that help companies and investors assess how sustainable and responsible a company is:

- **Environmental**: How well does a company manage its environmental impact? This includes issues such as pollution, energy consumption, and climate change.
- **Social:** How does a company treat people? This covers aspects like workers' rights, diversity, and community impact.
- **Governance:** How is the company organised and governed? This includes transparency, ethical behaviour, and the role of the board of directors.

Together, these factors support responsible investment decisions and promote sustainable business practices.

How does the Belgian biopharmaceutical sector perform in terms of sustainability?*

- 40% of companies consider a sustainable strategy essential, and 30% have implemented a formal sustainability strategy.
- Approximately 50% of companies have strongly integrated their social role and impact into their (sustainable) strategy.
- 50% of companies have already started carbon reduction initiatives, and around 25% have begun calculating their emissions.
- Around two-thirds of the surveyed companies have a dedicated ESG person within the organisation.
- Over half of the companies surveyed indicate that sustainability plays an significant role in securing capital financing.

** These findings are based on PwC's "Sustainability Survey in the Healthcare Space," which included responses from 41 companies in the healthcare industry, 17 of which were biopharmaceutical companies. The percentages reflect the responses specifically from the biopharmaceutical companies.*

What are the ESG challenges for the biopharmaceutical sector?**

Starting this year, Europe is imposing new sustainability reporting requirements on larger companies through the Corporate Sustainability Reporting Directive (CSRD). This means that these companies are required to consider integrating sustainability into their strategy and report on their ESG performance in alignment with the CSRD reporting framework. They will need to disclose how they integrate sustainability into their operations, the goals they set, and how they perform relative to those goals.

The biopharmaceutical sector has a very strong and positive social impact through the development of life-saving medicines, improving public health, and enhancing quality of life. Now, the sector is also increasingly taking responsibility for environmental issues. One example is the calculation of the carbon footprint. Many biopharmaceutical companies in Belgium already measure the carbon footprint of their own operations. However, only about a quarter extend this calculation to their entire value chain, referred to as scope 3 emissions. Under the CSRD reporting requirements, all larger companies will need to report on these scope 3 emissions, highlighting the responsibility companies must take for their entire value chain.

There is still progress to be made to bridge the gap between social and environmental impact. Biopharmaceutical companies must ensure they continue to save lives with innovative and essential medicines and vaccines while keeping their ecological footprint as small as possible across their value chain—from research and development to production, logistics, and patient use.

Today, many biopharmaceutical companies are already taking steps to reduce their emissions: greening vehicle fleets, buildings, and infrastructure, installing solar panels, sourcing green energy, and so forth. However, stakeholder pressure on sustainability will only increase in the coming years, and it's important to note that this pressure extends beyond the positive social role that biopharmaceutical companies already play. Smaller companies will also feel this pressure and need to improve their ESG performance as the entire ecosystem moves towards a more sustainable approach.

All these efforts must be made without compromising safety requirements. In general, over the next decade, the industry in Belgium and Europe will likely see a more balanced emphasis on both social impacts and environmental sustainability. Moving forward will require a joint, multi-sectoral approach to ensure that the industry can continue its life-saving mission in an environmentally responsible way.

***Based on an interview with Samar Héchaimé, Director of Sustainability Strategy & Transformation at PwC, and Thomas De Cuyper, Senior Director of Assurance at PwC*

Sustainability as a priority: pharma.be and its members building a green future

Europe is making significant strides to become climate-neutral by 2050, partly through the European Green Deal. These ambitious goals affect not only energy and transport sectors but also impact the pharmaceutical industry. While our members have already made substantial efforts to reduce their ecological footprint, new and proposed European regulations on sustainability could greatly impact our sector, including legislation on chemical substances, water purification, and product circularity.

As an umbrella organisation, we fully support the aim for a more sustainable world. Protecting both public health and the environment is one of our top priorities. There are challenges, however: the impact of these (non-pharmaceutical) regulations on our sector can extend across the entire pharmaceutical value chain. This could inadvertently affect aspects such as innovation, competitiveness, and even the availability of medicines in Belgium and Europe.

To better understand how these new regulations influence our sector, we review ecological sustainability issues within a dedicated focus group of experts from our member companies. Together, we work on solutions that benefit both the environment and public health.

With our combined efforts, we continue to contribute actively to a sustainable future and help build a healthy, green Europe.

Our ambition is to be a sustainable organisation, respected for the value it brings to society. We think long-term, understanding that our future depends on a healthy environment, a healthy society, and a robust economy. By driving change to combat diseases while striving for zero environmental impact, we address some of the most significant threats to human health and prosperity.

Sustainability is a core requirement at the heart of everything we do, guiding our ambition to positively impact society and ensuring our long-term success.

Our goal is 100% socially oriented, forming the foundation of our culture.

How do biopharmaceutical companies in Belgium view ESG?

We aim to foster sustainable behaviour among our employees not through enforcement or policy but by embedding it as a cultural value. We set a good example, for instance, by increasing the use of electric transport and consolidating orders to avoid separate deliveries of small batches.

Our sustainability strategy aligns closely with our business strategy, both directed toward social responsibility and impact, with patients at the centre.

As part of our mission to bring science to society, we must fully understand and acknowledge the broader social impact of our activities.

4.1.5 Caring for people and animals

Limiting animal testing[71]

The 3Rs are fundamental principles in animal research, requiring researchers to **reduce** the number of animals used in experiments, **refine** practices to minimise animal suffering, and, where possible, avoid or **replace** animal research with non-animal methods. The biopharmaceutical industry is strongly committed to upholding these principles.

In 2022 (the latest year for which data is available), 430,671 animals were used in research, a reduction of 1.5% compared to 2020. Among the animals used, 56.1% were mice and 14.6% were rabbits. Dogs and cats were used to a much lesser extent (0.08% and 0.05%, respectively).

In 2022, the majority of laboratory animals were used for fundamental and applied research (77%). In 2020, this share was 66.2%. Just under 100,000 laboratory animals, or 23% of the total number were used in the context of regulatory studies and routine production (quality and efficacy testing toxicity tests, etc.), marking a reduction of nearly 30,000 animals.

Number of animals used in research

Source: EU Statistical Data of all users of animals

More rational use of antibiotics in animals

Ensuring that medicines are used properly is an important objective for pharma.be and its members. One of the initiatives taken by the medicine industry in 2011 was the establishment of **AMCRA**, the knowledge centre on antibiotic use and resistance in animals (see box).

Within AMCRA, pharma.be works together with the faculties of veterinary medicine of the Universities of Ghent and Liège, farming organisations, veterinarians and feed manufacturers to promote the rational use of antibiotics in animals based on the principle: **"as much as necessary, as little as possible."**

By creating guidelines, benchmark reports for veterinarians and information campaigns for farmers and the general public, we have succeeded in significantly reducing the use of antibiotics in animals without negatively impacting the health and welfare of agricultural and domestic animals.

The figures on antibiotic use in 2023 show that we are clearly on the right track with a total use of 55.0 mg of antibiotics per kilogram of biomass.This is a decrease of 21.7% compared to 2022, but more importantly an overall decrease of 62.4% if we compare it with 2011. Among critical antibiotics, we have also seen a decrease of more than 75% since the start of our efforts.

To continue this progress, AMCRA has developed a new vision for the 2025-2030 period, building on past efforts with clear objectives and concrete actions.

Together with veterinarians and livestock farmers, we aim to bring the use of antibiotics in animals down to the European median by 2030 and significantly reduce the use of antibiotics that are essential for human treatment.

This involves continuing to collect data on antibiotic use in animals, supporting livestock farmers, promoting prevention, and encouraging the use of alternative therapies.

pharma.be and its members will therefore remain a loyal partner within AMCRA in the future. We will continue to work with all relevant stakeholders to ensure that both animals and humans can continue to rely on high-performing antibiotics when they need them.

Who or what is AMCRA?

AMCRA stands for Antimicrobial Consumption & Resistance in Animals. AMCRA is a federal knowledge centre dedicated to the reduction of antibiotic resistance and antibiotic use in animals in Belgium. The organisation collects and analyses relevant data and uses this data to raise awareness among veterinarians and animal owners and provide targeted advice.

It is no coincidence that pharma.be's Animal Health Group was one of the co-founders of AMCRA in 2012. AMCRA's objectives largely reflect the vision of our own organisation.

pharma.be aims to ensure that sick animals in Belgium can be treated with high-quality veterinary medicines. The One Health concept is key in this respect: animal health, human health and environmental health are all closely connected and affect one another. Sustainable use of antibiotics in veterinary medicine is therefore important to help safeguard public health.

4.2 Working together for the benefit of the patient

4.2.1 Working together with patient organisations

From Patient to Partner

At pharma.be, patients are at the heart of all operations, with the goal of ensuring that **patients** have the **best possible access to therapeutic innovation**. This is achieved not only for patients but, more importantly, in collaboration with them. Only in this way can this goal be fully realised. The 2023 Roundtable for Patient Organisations was therefore aptly titled 'From Patient to Partner.'

Mission & Objectives

In spring 2021, pharma.be launched the Patient Engagement working group, consisting of patient ambassadors and those responsible for patient engagement within pharma.be member organisations. Together, they set the key focus areas each year.

For 2024, the working group outlined a clear **mission**:

To systematically integrate the patient's voice into the healthcare system and become a trusted partner for collaboration, guided by our 10-point checklist.

The 10-point checklist, developed in 2023 with patient input, serves as a framework for pharmaceutical companies to work in a patient-centred manner.

This mission for 2024 has been translated into three concrete objectives:

1. Supporting patient health literacy and further simplifying administration for patient organisations
2. Improving collaboration between companies and patient organisations
3. Systematically integrating the patient perspective into operations

From dialogue ...

The mission and objectives result from intensive listening and collaboration with patient organisations, making dialogue with these organisations a cornerstone for pharma.be. Just as the 10-point checklist offers members a framework for patient-centred approaches, pharma.be uses various methods to facilitate this dialogue. The Patient Engagement working group is one such method, and the Patient Advisory Board is another. However, perhaps the most prominent is the annual Roundtable for patient organisations, a highlight each year where knowledge is shared, listening is prioritised, and intensive debates are held. The invaluable insights from this Roundtable then serve as the foundation for action within the Patient Engagement working group. The **third Roundtable on 21 October 2024** once again provided numerous action points, with over 50 patient organisations participating. Under the theme 'Patient Engagement is a Right and a Responsibility,' a comprehensive programme was offered,

including a review of the previous Roundtable's outcomes and presentations on the latest developments and hot topics:

- The added value of patient organisations
- Patient Empowerment Charter
- Health literacy
- The patient's voice in the new reimbursement procedure
- The importance of health data.

Following this, smaller groups engaged in intensive discussions on how the patient's voice can be more fully heard in the broader healthcare system and where there may be further or new opportunities for collaboration.

... to action

These Roundtables go beyond informing, debating, and networking; they primarily serve as the foundation for developing concrete actions. In 2024, two projects were launched to empower patients further:

1. **Consultancy Agreement**

   The Roundtable on 28 November 2023 highlighted the need for the biopharmaceutical industry to simplify administration for patient organisations. Many patient organisations lack the time, expertise, or manpower to review or draft extensive contracts.

   In response, pharma.be, together with industry members, developed a simplified 'Consultancy Agreement' model. Such agreements can often exceed fifty pages, but in the simplified version, the essential clauses were condensed into a three-page document—a significant improvement. Despite its brevity, the new agreement meets all criteria to comply with the highly regulated biopharmaceutical sector.

   The simplified agreement was validated by both pharma.be members and patient organisations, as well as the Patient Advisory Board.

Patient organisations are essential allies in the healthcare landscape, advocating for positive changes that benefit all stakeholders and society at large. Their voices and perspectives are now more critical than ever to drive continuous innovation within the health system.

Sofie Bekaert, Head of Programme, Health, King Baudouin Foundation

pharma.be Patient Advisory Council

To help the Patient Engagement working group prioritise and evaluate its projects, pharma.be relies on a Patient Advisory Board.

The advisory board has 9 members:

- Inge Van de Velde (MS)
- Veerle De Pourcq (ReumaNet)
- Elke Stienissen (Lymfklierkanker)
- Axel Vanderperre (HIV)
- Eva Schoeters (RadiOrg)
- Stefan Joris (Muco)
- Gay Charles (MyMu)
- Veronique Van Assche (SMA)
- Katleen Franc (Crohn & Colitis)

The Patient Advisory Council discusses not only current but also future needs. Together, we determine the actions through which we can create the greatest impact, aiming to foster greater collaboration among companies to enhance quality outcomes for patient organisations while simplifying processes for them.

View the template

2. A Guide to Better Collaboration

Another concrete action to facilitate collaboration is a handbook for partnerships between biopharmaceutical companies, patient organisations, healthcare providers, and other organisations. As collaboration increases, which is positive, there remains a lack of synergy between biopharmaceutical companies on shared projects. This gap limits opportunities to make projects even stronger and more sustainable.

Therefore, pharma.be is developing a collaboration guide for the entire patient journey. The guide will provide a theoretical framework along with numerous examples to inspire current and future partnerships, including practical tips and tricks for optimising collaboration. It will be structured as a "living" document, allowing for new examples and lessons learned to be added over time.

Through such concrete actions and systematic dialogue, pharma.be aims to continue creating value for patients and patient organisations, working towards solutions that deliver the best possible healthcare. Together, we are building a future where the patient's voice is firmly embedded within the healthcare system.

4.2.2 Working together for relevant health data

What are Real World Data and Real-World Evidence?

Real World Data (RWD) is an umbrella term for data on the effects of health care (such as safety or effectiveness) that is not collected in the context of highly controlled randomised clinical trials (Randomised Clinical Trial, RCT). These may include, for example, clinical and economic results, administrative data, patient-reported outcomes (PROs) and health-related quality of life (HRQoL). These data come from very diverse sources, such as patient registries, electronic medical records or health insurance company databases.

According to the US FDA, Real World Evidence (RWE) refers to clinical evidence regarding the usage and potential benefits or risks of a medical product derived from analysing RWD. RWE complements traditional clinical trials to enable validation in daily clinical practice.

The Added Value of Real-World Data

In primary and secondary use

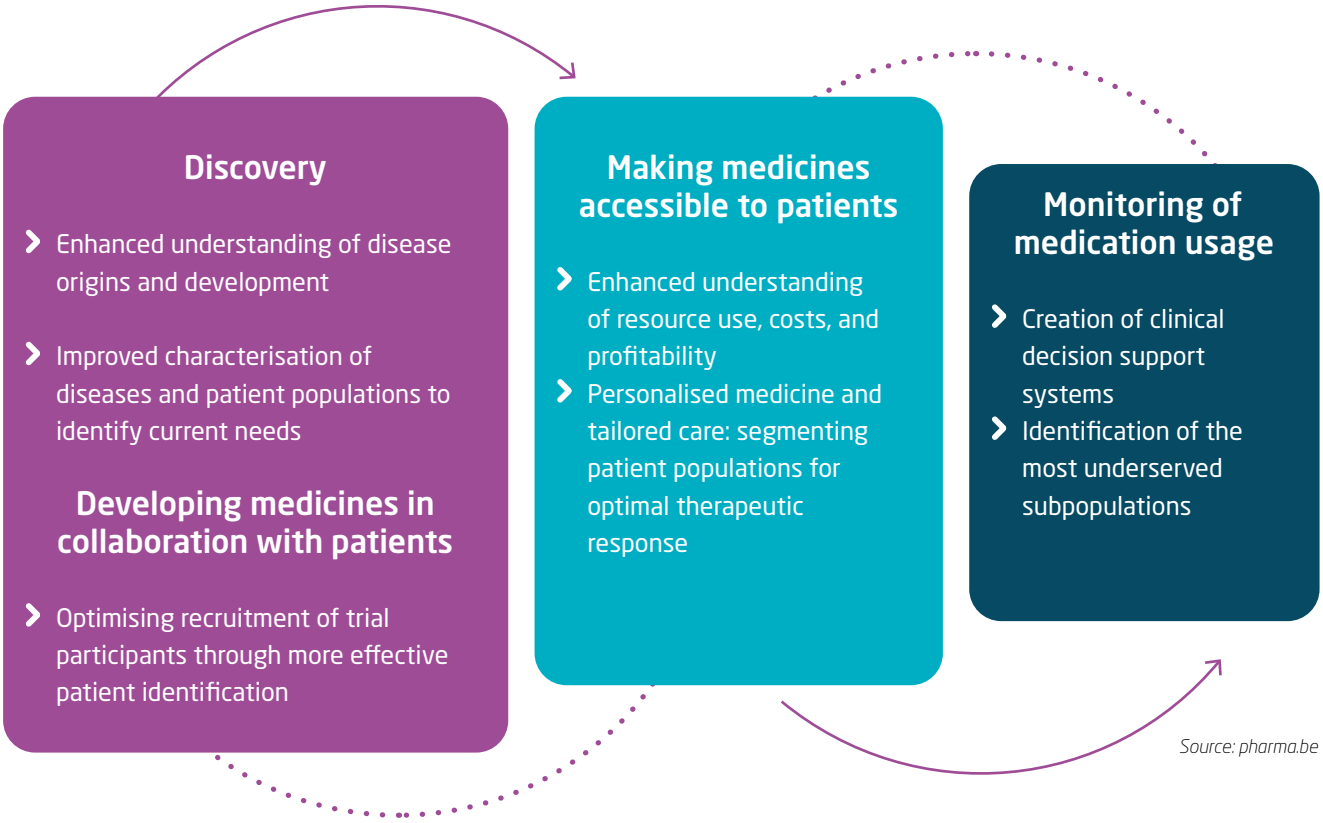
To provide each patient with the best possible care, a substantial amount of data is collected. This may include information on allergies, medications used, previous surgeries, scan results, blood tests, and so on. These data are shared with the patient and their healthcare providers, enabling all parties to work together effectively on the patient's health or recovery. This constitutes the primary use of health data.

The health data of individual patients can also provide valuable insights for other or future patients. By sharing and analysing a wide range of data, healthcare providers can design improved care pathways, accelerate medical research into new treatments, and identify patterns and connections that may not be visible in individual cases. These insights are crucial for developing efficient and effective healthcare strategies. This secondary use of health data contributes to the continuous improvement of care and our healthcare system.

In the life cycle of a medicine

Throughout a medicine's lifecycle, health data are invaluable—from the earliest discovery phase to the post-market surveillance phase when a new medicine is eventually available to the public. Real-World Data (RWD) contributes to a better understanding of disease development and progression, facilitates patient recruitment for clinical trials, enhances cost and cost-effectiveness insights, supports evidence-based medicine, and more.

This diagram provides an overview of the added value of using RWD throughout the lifecycle of a medicine:

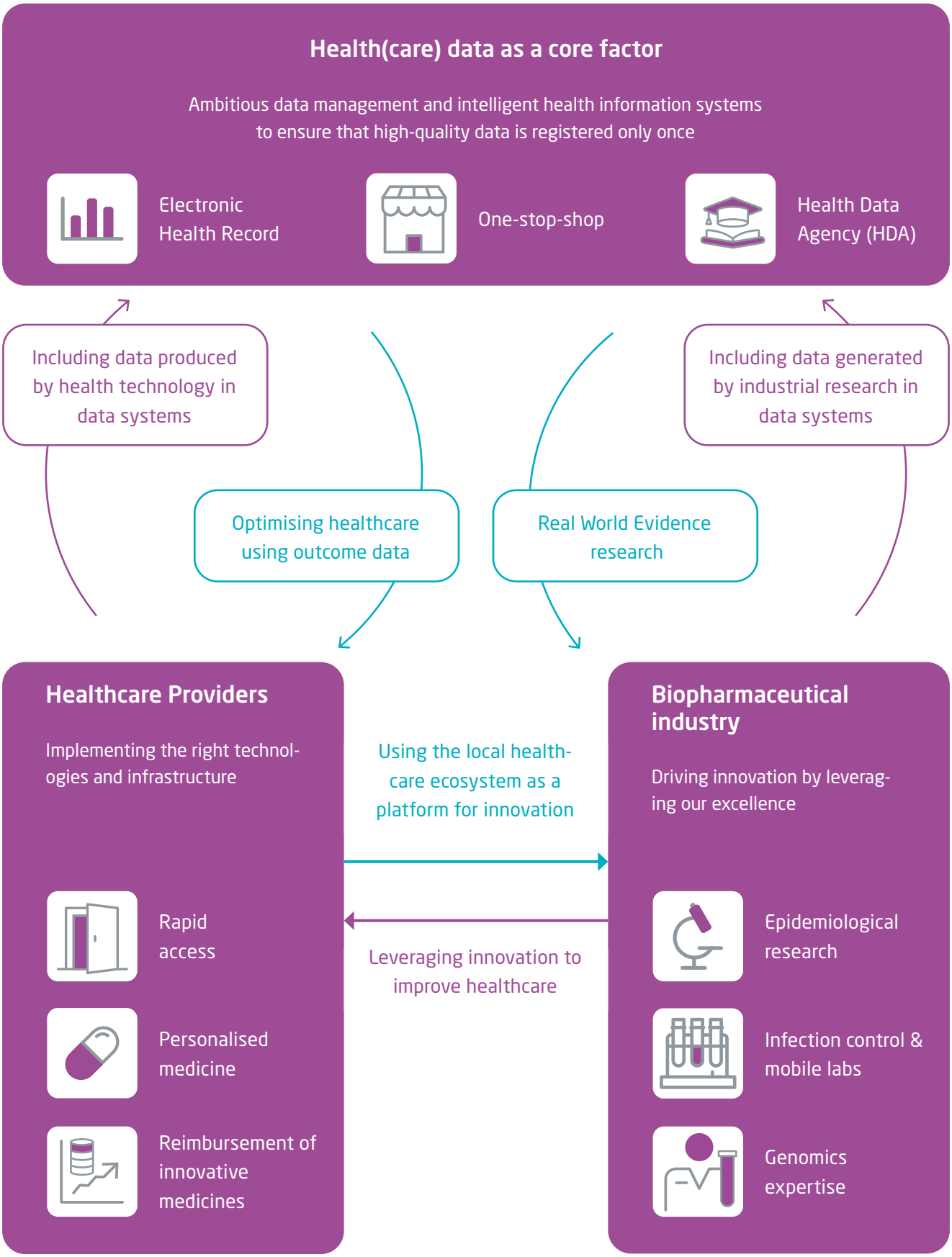


Source: pharma.be

A health data ecosystem

To effectively use RWD for innovative treatments that improve patient quality of life, a well-functioning ecosystem is essential. This begins with the foundation: individual patient health data, which must meet specific criteria to be suitable for reuse. Data collection and processing must also protect patient privacy at all times.

However, data alone is insufficient. To fully realise the potential of RWD, clear commitments and agreements are needed among core stakeholders: healthcare providers and the biopharmaceutical industry. They must also have the required expertise and (technological) infrastructure, supported as necessary by external service providers. The government plays a crucial role in the ecosystem by providing a regulatory framework and incentives to encourage the reuse of RWD to improve healthcare.


Health(care) data as a core factor
Ambitious data management and intelligent health information systems to ensure that high-quality data is registered only once
Electronic Health Record
One-stop-shop
Health Data Agency (HDA)
Including data produced by health technology in data systems
Including data generated by industrial research in data systems
Optimising healthcare using outcome data
Real World Evidence research
Healthcare Providers
Implementing the right technologies and infrastructure
Rapid access
Personalised medicine
Reimbursement of innovative medicines
Using the local healthcare ecosystem as a platform for innovation
Leveraging innovation to improve healthcare
Biopharmaceutical industry
Driving innovation by leveraging our excellence
Epidemiological research
Infection control & mobile labs
Genomics expertise

Privacy protection

RWD is incredibly valuable but also highly sensitive, as it involves deeply personal patient information. In both primary and secondary uses of RWD, **patient privacy protection remains paramount**. Health data may only be reused if the General Data Protection Regulation (GDPR) and Belgian privacy laws are respected.

Data must, under no circumstances, be traceable to an individual patient. A key method to ensure this is federated analysis, whereby data remains with the original sources, and researchers only access aggregated and anonymised analysis results, not the raw data itself, minimising privacy risks.

FAIR Data

A federated approach requires data that is **Findable, Accessible, Interoperable, and Reusable (FAIR)**. Without data meeting these FAIR principles, researchers and developers cannot proceed effectively, and RWD cannot provide added value.

A 'health data' ecosystem

FAIR data is at the basis of a health data ecosystem. FAIR stands for:

F

Findable

to be usable, data must of course be easy to find, for example via a metadata catalogue in one place.

A

Accessible

data must be accessible (within a predefined and transparent framework).

I

Interoperable

databases and collections must be compatible with our Belgian e-health infrastructure so that they can be linked together.

R

Reusable

data should be reusable for research, healthcare and policy decisions.

Belgium's RWD and Digitalisation Policy

Belgium has made significant progress in making health data FAIR, thanks in part to the Health Data Agency, established in March 2023 to facilitate the use of health data in scientific research. One of its core activities is ensuring data meets FAIR standards, making Belgium a European frontrunner in this area.

The NIHDI also contributes to this leadership position with initiatives like the learning network RWE4Decisions, which brings together stakeholders – including patient organisations, government bodies, the biopharmaceutical industry, and data experts – to determine who, when, and how RWD should be collected to generate Real-World Evidence (RWE) that supports patients, healthcare providers, and healthcare systems in decision-making.

Belgium also excels in healthcare digitalisation, ranking number one in e-health and data infrastructure.

Digitalisation encompasses a wide range of technologies that enable doctors to electronically record health data, follow up on patients, and more. Through mHealthBELGIUM, Belgium is a pioneering country in Europe for supporting healthcare technology, laying the groundwork for telemedicine and remote monitoring.

pharma.be and RWD

With these strengths, Belgium is well-positioned to build a robust health data ecosystem and to maintain its leading role in clinical trials while striving for a global top position in RWD research. However, there is no time to lose in implementing this ambitious data policy, and pharma.be is (pro)actively engaged in this effort across various areas. **Biopharmaceutical research with RWD leads to new health outcomes that benefit patients.**

For nearly a decade, pharma.be has had an RWD focus group and, since 2022, a Health Data & Digitalisation taskforce. This taskforce supports or initiates projects and policy initiatives in health data and digitalisation. One of the taskforce's key initiatives is the development of guidelines and a step-by-step plan to align RWD projects correctly with GDPR and Belgian privacy laws.

The results of these initiatives are also discussed at the annual Forum Health Data & Digitalisation hosted by pharma.be. This forum promotes open debate among RWD and digital healthcare experts, companies, government bodies, and other stakeholders. Additionally, pharma.be supports government initiatives, such as those by the Belgian Health (Care) Data Agency, to ensure that RWD can be reused safely and ethically. The biopharmaceutical industry has amassed extensive experience and knowledge in data projects and is eager to share this expertise.

Additional value of RWD with AI

pharma.be looks forward to continuing the digital transformation of Belgium's healthcare ecosystem with its partners, aiming to enhance patient health outcomes. Numerous new opportunities are on the horizon, one of the most significant and transformative being **artificial intelligence (AI)**. While the actual added value of AI for healthcare was not immediately clear in the early stages of this technology, it has since been **proven that integrating AI into the biopharmaceutical sector can radically transform how medicines are developed, tested, and delivered.**

One of AI's greatest advantages is accelerating the medicine development process.

Currently, developing a new medicine is a lengthy process with limited success rates and high costs. However, AI algorithms now allow for the analysis of vast datasets, enabling faster identification of potential medicine candidates.

AI also plays a crucial role in improving clinical trials. By using AI, biopharmaceutical companies can more rapidly identify suitable patients, significantly shortening recruitment times. AI assists in monitoring patients during clinical trials and in analysing the vast amounts of data collected throughout these studies. This not only leads to more efficient research but also to better and more reliable results.

AI also advances personalised medicine significantly. By analysing patient data and recognising patterns, AI systems can develop tailored treatments that align better with individual patient needs, leading to more effective treatments with fewer side effects. In Belgium, AI is already being utilised to develop patient-specific treatments, particularly in complex disease areas such as cancer and rare diseases.

New opportunities, however, bring new concerns—or rather, familiar concerns revisited in this new context. Protecting patient data and managing data and new technologies ethically have always been priorities for pharma.be, and this focus remains stronger than ever. We aim to be a highly reliable data partner and a responsible data steward within the wider healthcare ecosystem.

4.2.3 Working together for the availability of vaccines and medicines

Medicines should always be available whenever patients need them. However, in reality, medicines are sometimes temporarily not available. This can be challenging for patients; however, biopharmaceutical companies are equally committed to ensuring their medicines are always available when needed. Fighting medicine shortages requires more than just the good will of stakeholders on the ground.

Situation in Belgium

Many initiatives are already being taken in Belgium in consultation with the Federal Agency for Medicines and Health Products (FAMHP) to supply wholesale distributors and pharmacists in time for patients to have their medicine. The FAMHP was the first in Europe to set up a reporting system, **"PharmaStatus"**, which provides comprehensive thorough transparency regarding the causes and duration of unavailability for the benefit of healthcare professionals and patients (see box). Companies also prudently stockpile medicines to supply pharmacists directly to ensure availability for Belgian patients.

However, unavailability is a complex issue with many causes at different levels.

Complex production process

First, pharmaceutical companies do not take any risk in terms of safety and quality of products. Inspections are also particularly strict in Europe. The manufacturing processes for medicines and vaccines are highly time-consuming, extraordinarily complex, and delicate. Most vaccines, for example, have a manufacturing process that takes more than 18 months. The same is true for other biological medicines developed from living cells. An unexpected issue at every step in this process, sometimes cannot always be resolved immediately or easily, potentially leading to major delays in the delivery of the next batch. With dire consequences, including stock-outs, often not only for the Belgian market but also for other European or even non-European countries. After all, medicines are rarely produced for one country, but for an entire region or even the world. Scaling up to meet increased demand cannot happen overnight. Raw materials are also often scarce and these need to be handled very carefully. Holding large reserve stocks is therefore not justified and could lead to major upward price pressure or even scarcity of other medicines that require the same raw materials.

Price pressure

Second, we note that maintaining local production in Europe is a major challenge for all industrial sectors. **Higher wage and energy costs mean that production at low prices is not competitive and those activities are shifting to other parts of the world.**

The biopharma sector is not exempt from this dynamic. Only high-value-added activities requiring extensive specialised knowledge are able to avoid it for now.

Moreover, frequent price cuts for medicines in the off-patent segment, imposed by the Belgian government, sometimes make it unfeasible for companies to continue offering certain medicines. As a result, these medicines may permanently disappear from the Belgian market, which can be highly disadvantageous for Belgian patients.

Free movement of goods

Finally, medicines must be able to circulate in Belgium according to the free movement of goods in the European Union. Since the price of a medicine is set by governments on a country-by-country basis, this often leads to the movement of medicines from countries with lower prices to those where they are sold at higher prices.. We call this **parallel export.** The quota system attempts to address this issue, but it is often challenging for biopharmaceutical companies to supply adequate quantities quickly in the event of an export leak. Scaling up production is a complex and time-consuming process.

Protecting patients from additional costs

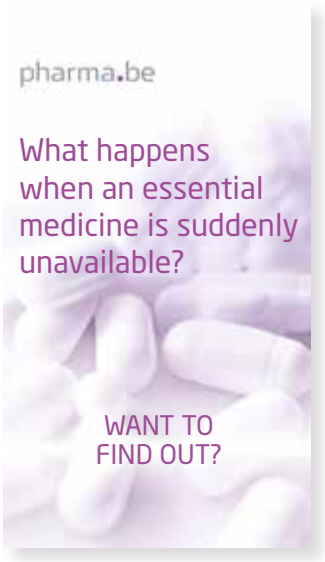
In 2024, a collaborative effort involving all stakeholders led to the development of a system designed to protect patients from incurring additional costs when their medicine is unavailable, and an alternative must be imported by pharmacists from abroad. In many cases, these imported medicines are more expensive and not reimbursed, resulting in significant costs for patients. However, through this system to which all biopharmaceutical companies contribute, patients will no longer face extra charges for an imported medicine that replaces an unavailable product.

Our commitment

The issue of unavailability is being discussed with the health minister as part of the reform plans. To make and keep medicines available to Belgian patients in the future, we will have to make the necessary long-term adjustments to make our distribution system less susceptible to all kinds of external factors. Encouraging a healthy competitive economic environment that allows multiple stakeholders into the Belgian market is one of the necessary adjustments to put a sustainable distribution system in place.

That is our commitment to society, a role we take very seriously as an industry.

That is why pharma.be, together with the FAMHP and all stakeholders within the distribution chain, is looking for solutions to minimise the impact of non-availability of a medicine on patients.



What does PharmaStatus tell us ?

Along with the other actors, the biopharmaceutical sector should be transparent and provide clear information on medicines that are not available. The FAMHP's online application PharmaStatus plays an important role in this. Thanks to PharmaStatus, doctors, pharmacists and patients can easily check how long their medicine will be unavailable and why. Through PharmaStatus, the FAMHP can also provide alternatives for unavailable medicines. Finally, using PharmaStatus, wholesale distributors, pharmacists and companies can work together to find solutions if a medicine is unavailable in a pharmacy or at a wholesale distributor.

Looking at the actual figures, we see that 590 medicines were temporarily unavailable in October 2024. Although this has an impact on the patient and the healthcare provider (getting a new prescription, additional visits to the pharmacy), the impact on the continuity of treatment is limited. In 393 cases there was at least one alternative available, and in 332 of these cases patients had even three or more alternatives. In a majority of the other cases, the patient could be helped by importing a medicine from abroad or by adjusting the treatment. Where even these solutions were not possible, the FAMHP convened a working group to formulate recommendations to ensure the care of affected patients.

4.2.4 Collaboration for the correct implementation of European regulations

European Health Technology Assessment (HTA) Regulation Effective from 12 January 2025

From 12 January 2025, the new European regulation for Health Technology Assessment (HTA) comes into force, initially focusing on new oncology products and advanced therapy medicinal products (ATMPs). Following the initial implementation, orphan medicines will be included in 2028, and other medicines will follow in 2030. This new regulation will bring significant changes to the evaluation of new medicines.

The new EU HTA procedure means that the assessment of new medicines will no longer occur solely on a national level but partly through a joint European process. This integrated approach between EU and national HTA will make more efficient use of resources, reduce duplication, and create greater predictability in the assessment process. Early involvement of all stakeholders is essential for optimal alignment.

Collaboration between NIHDI, the Commission of Reimbursement of Medicines (CRM), biopharmaceutical companies, patients, and clinicians is crucial to successfully implement this new regulation. The Joint Clinical Assessments (JCA) will improve quality and expedite decision-making at the national level. This avoids repeated evaluations at the national level, accelerating access to innovative technologies for Belgian patients.

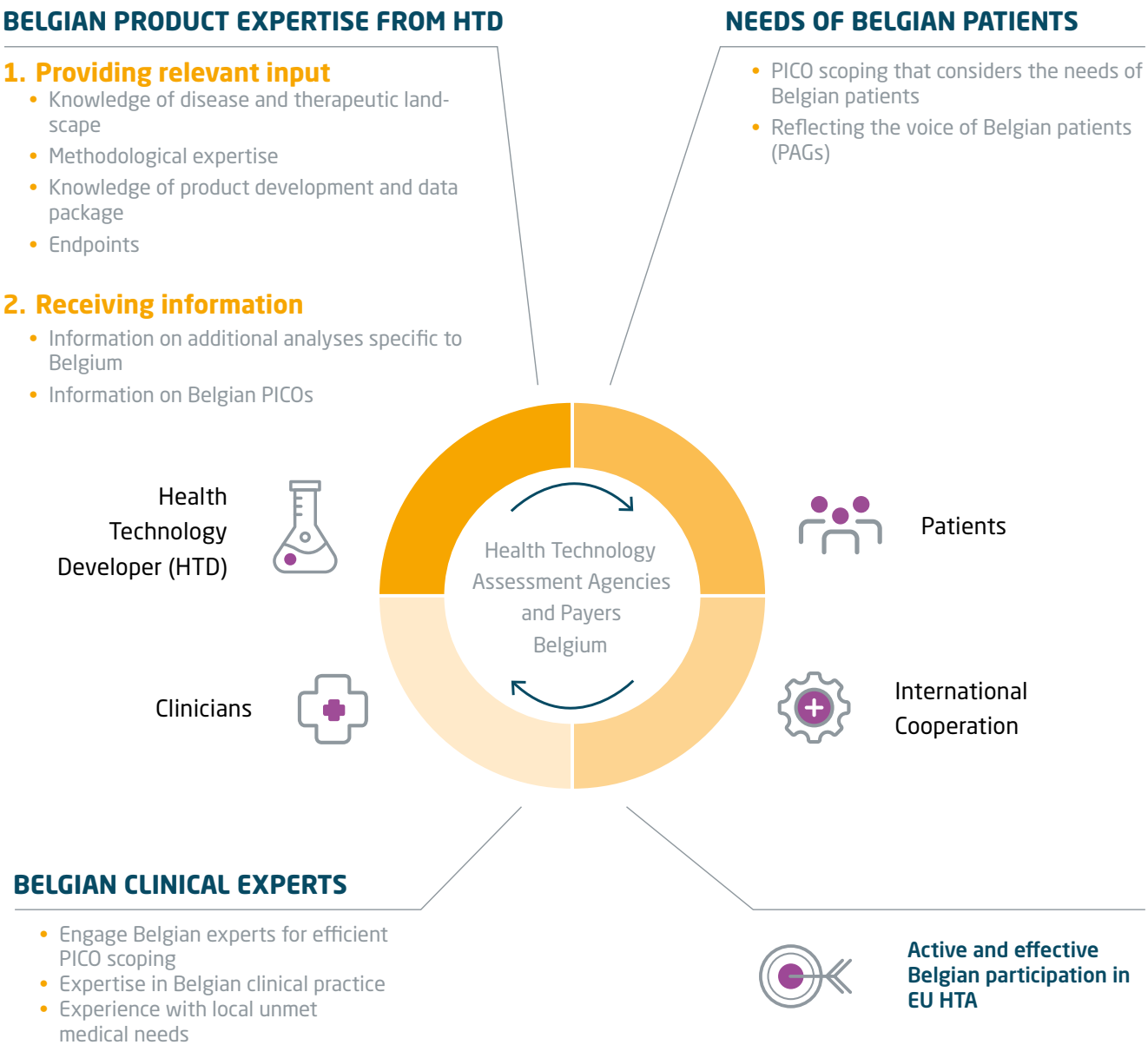
On 30 January 2024, the European Commission and the Heads of HTA Agencies Group (HAG), along with national HTA agencies from the Netherlands, Austria, Belgium, Ireland, and Luxembourg, hosted a meeting focusing on the national implementation of this regulation.

Key stakeholders attended, including NIHDI and representatives from patient associations, healthcare professionals, scientific associations, the industry, national decision-makers, and regional health authorities. pharma.be participated in the panel "Ensuring Engagement and Cooperation in Joint Clinical Assessments", emphasising the importance of collaboration from the start of joint clinical assessments.

On 30 September 2024, pharma.be, in collaboration with NIHDI and KCE, organised an information session on the practical implementation of this new European regulation at the national level. In the context of the European HTA Regulation, NIHDI (National Institute for Health and Disability Insurance) and KCE (Federal Centre of Expertise for Health Care) have specific roles within the Coordination Group. NIHDI oversees the process and evaluation of new treatments for reimbursement, while KCE provides scientific advice and supports the assessment of new therapies by developing research methodologies. Both organisations work closely together to ensure an efficient evaluation process and timely access to medicines for patients in Belgium.

Early consultation with all stakeholders allows for the pooling of expertise, knowledge, and insights, ensuring that Belgian patients gain timely access to effective, safe, and high-quality new medicines.

Proactive Belgian collaboration will prevent duplication of efforts for all stakeholders, resulting in efficient EU and Belgian HTA processes



Belgium: A pioneer in the rollout of the European Falsified Medicines Directive

On 9 February 2019, the European Falsified Medicines Directive (FMD) came into effect. Since then, most prescription medicines must be equipped with an *anti-tampering device*, allowing for checks on whether packaging has been opened. Medicines must also carry a unique, two-dimensional matrix code.

The marketing authorisation holder activates this code through a secure European network, storing it in a database for tracking during the distribution process. Full-line wholesalers verify the code upon delivery if they do not purchase directly from the manufacturer. When dispensing the medicine to a patient, the (hospital) pharmacist scans the code once more for a final check and deactivation.

Full-line wholesalers and pharmacists who encounter an error message during verification must isolate the product until the authenticity of the medicine is confirmed or disproven. This comprehensive control system plays a crucial role in protecting the supply chain against counterfeit and fake medicines.

Belgium led the way in the European Union in rolling out the FMD. By 9 February 2024, exactly five years after its introduction, the FMD had been fully implemented in Belgium, thanks mainly to the efforts of the Belgian Medicines Verification Organisation (BeMVO). This organisation is responsible for managing the technical system for tracking and validating the unique codes on medicine packaging in Belgium.

As a member of BeMVO's governing body, pharma.be supports these essential measures alongside other partners in the medicine supply chain. Strong collaboration is crucial to prevent counterfeit medicines from reaching patients.

With the full implementation of the European Directive, we are taking a significant step forward in ensuring the safety of our medicines. As one of the leaders in Europe, we can be proud of this achievement as a sector.

Philippe Coene, General Manager BeMVO

APPENDIX 1 - METHODOLOGY OF COST-BENEFIT ANALYSIS

In section 3.2.2, we summarised the cost-benefit analysis of the biopharmaceutical industry's impact on the Belgian government. Below is a detailed breakdown of the expenditures and income that formed the basis of our analysis.

Detailed breakdown of expenditure (in thousand euros)

Cost for the government (2023)	4,459,675
State expenditure on medicines (industry costs, excluding VAT) - NIHDI	4,360,013
1.2. Subsidies	99,662

State expenditure on medicines is based on the NIHDI figures of expenditure on specialty biopharmaceuticals. This expenditure is composed of the ex-works price of medicines, distribution costs and VAT. In this analysis, we only take into account the ex-works price, excluding distribution costs and VAT.

The subsidies paid by the government to the biopharmaceutical industry are derived from the annual accounts of companies operating in Belgium. These figures are sourced from the following headings: 740 (operating subsidies and compensatory amounts received on behalf of the government), 9125 (capital subsidies granted by the government), and 9126 (interest subsidies granted by the government).

Detailed income statement (in thousands of euros)

Income for the government (2023)	5,939,843
2.1. Labour charges	2,762,516
2.1.1. Employer's social security contribution	964,401
2.1.2. Employee's social security contribution	523,229
2.1.3. Retained amounts charged to third parties as tax on wages and salaries	1,274,887
2.2. Corporate tax	418,459
2.3. Taxes	1,643,181
2.3.1. VAT on turnover (6% ex-works price non-reimbursed medicines)	198,486
2.3.2. NIHDI taxes on turnover	444,553
2.3.3. Amounts retained on behalf of third parties for tax on income from investments	84,906
2.3.4. Corporate taxes and levies	915,235
2.4. Indirect revenues from purchases from third parties and from investments	1,115,687
2.4.1. Purchase of raw materials and goods, various goods, and services	1,028,975
2.4.2. Investments	86,712

The income from labor charges is derived from the annual financial statements of companies operating in Belgium. These include the following headings: 621 (employer's social security contributions), 620 (remuneration and direct social benefits, NSSO part), and 9147 (withholding tax). The same approach applies to corporate tax, which corresponds to heading 670 (taxes).

The taxes are divided into four elements:

1. VAT on non-reimbursed medicines
   The calculation uses the turnover figure for non-reimbursed medicines as reported by IQVIA. We do not take into account VAT on reimbursed medicines because the NIHDI pays it to the government, so it does not affect the comparison.

2. Taxes paid to NIHDI by companies based on their turnover
   This figure is provided by NIHDI.

3. Balance sheet item 9148 (withholding tax)

4. Balance sheet item 640 (corporate taxes and levies)

Besides direct revenue, there are also indirect revenues for the government:

1. Revenues from domestic purchases of raw materials, goods, various goods, and services by the biopharmaceutical industry

   The calculations are based on data from the input-output tables provided by the Federal Planning Bureau, which detail the domestic demand of the biopharmaceutical sector on other sectors. For each sector, the ratio of value added to turnover (also available in the input-output tables) is applied to this domestic demand. This is then adjusted by the average (para)fiscal levy rate of 42.43% (OECD).

2. Revenues from investments by the biopharmaceutical industry

   We apply the ratio of value added to turnover in the manufacturing industry to the amount invested, based on data from Statbel. The average (para)fiscal levy rate is then applied (42.43%, OECD).

APPENDIX 2 - ABBREVIATIONS

- AMCRA: Antimicrobial Consumption & Resistance in Animals
- ATC: Anatomical Therapeutic Chemical
- ATMP: Advanced Therapy Medicinal Product
- BCFI: Belgisch Centrum voor Farmacotherapeutische Informatie, Belgian Centre for Pharmacotherapeutic Information
- BTGC: Bureau van Toezicht op de Geschreven Communicatie, Bureau for Control on Written Communication
- DEF Committee: Commissie voor Deontologie en Farmaceutische Ethiek, Deontology and Pharmaceutical Ethics Committee
- CMR: Commissie Tegemoetkoming Geneesmiddelen, Commission of Reimbursement of Medicines
- CUP: Compassionate Use Programme
- DALY: Disability Adjusted Life Years
- EFPIA: European Federation of Pharmaceutical Industries and Associations
- EMA: European Medicines Agency
- E-PIL: Electronic Patient Information Leaflet
- ETA: Early Temporary Authorisation
- FAMHP: Federal Agency for Medicines and Health Products
- FAIR: Findability, Accessibility, Interoperability, and Reusability
- FDA: Food and Drug Administration
- HRQoL: Health Related Quality of Life
- IFPMA: International Federation of Pharmaceutical Manufacturers & Associations
- MIDAS: Migraine Disability Assessment
- MNP: Medical Need Program
- OECD: Organisation for Economic Co-operation and Development
- R&D: Research and Development
- PEC: Patient Expert Center
- PO: Patient Organisation
- PRO: Patient-Reported Outcomes
- QALY: Quality-Adjusted Life Year
- RCT: Randomized Controlled Trial
- NIHDI: National Institute for Health and Disability Insurance
- RWD: Real World Data
- RWE: Real World Evidence
- SmPC: Summary of Product Characteristics
- Statbel: the Belgian statistical office
- STEM: Science, Technology, Engineering & Mathematics

References

1 Source: Regulation No 1901/2006, https://eur-lex.europa.eu/legal-content/EN/TXT/HTML/?uri=CELEX:32006R1901
2 Source: https://www.uantwerpen.be/nl/centra/universiteitsfonds/maak-mee-het-verschil/doe-een-gift/onze-leerstoelen/antwerp-pediatric-clinical-trial-network/
3 Source: https://www.bpcrn.be/network-updates/bpcrn-kick-off-meeting-september-6th-2024-uz-gent
4 Source: https://www.sciensano.be/sites/default/files/vaccine_coverage_2020-21_nl_final.pdf
5 Source: https://www.phrma.org/Scientific-Innovation/Progress-in-Fighting-Rare-Diseases
6 Source: https://www.riziv.fgov.be/nl/thema-s/verzorging-kosten-en-terugbetaling/wat-het-ziekenfonds-terugbetaalt/geneesmiddel-en/geneesmiddel-terugbetalen/weesgeneesmiddelen
7 Lichtenberg, 2022
8 Seboio, 2020
9 EFPIA, 2019
10 Lichtenberg, 2019
11 EFPIA, 2019
12 EFPIA, 2022
13 Seboio, 2020
14 Wassenberg T, Molero-Luis M, Jeltsch K, et al. Consensus Guideline for the diagnosis and Treatment of Aromatic L-Auimino Acid Decarboxylase (AADC) Deficiency. Orphanet J Rare Dis 2017;12(1):12
15 Mangeold C, Hoffmann G, Degen I, et al. Aromatic L-Amino Acid Decarboxylase Deficiency: Clinical Features, Drug Therapy and Follow-Up. JIMD. 2009; 32: 371-380
16 Summary of Product Characteristics, 11 March 2024, (the link can be accessed at info@pharma.be)
17 Tai CH, Lee NC, Chien YH, Byrne BJ, Muramatsu SI, et al. Long-term efficacy and safety of eladocagene exuparovovec in patients with AADC deficiency. Mol Ther. 2022; 30(2): 509-518
18 Chen, T., D. Wu, H. Chen, W. Yan, D. Yang, G. Chen, K. Ma, D. Xu, H. Yu, H. Wang, T. Wang, W. Guo, J. Chen, C. Ding, X. Zhang, J. Huang, M. Han, S. Li, X. Luo, J. Zhao and Q. Ning (2020)."Clinical characteristics of 113 deceased patients with coronavirus disease 2019: retrospective study." Bmj 368: m1091
19 Cummings, M. J., M. R. Baldwin, D. Abrams, S. D. Jacobson, B. J. Meyer, E. M. Balough, J. G. Aaron, J. Claassen, L. E. Rabbani, J. Hastie, B. R. Hochman, J. Salazar-Schicchi, N. H. Yip, D. Brodie and M. R. O'Donnell (2020). "Epidemiology, clinical course, and outcomes of critically ill adults with COVID-19 in New York City: a prospective cohort study." Lancet 395(10239): 1763-1770
20 Parasher A. 2021. COVID-19: Current understanding of its Pathophysiology, Clinical presentation and Treatment. Postgrad Med J 97:312-320. DOI: 10.1136/postgradmedj-2020-138577
21 Harvey WT, et al. 2021. SARS-CoV-2 variants, spike mutations and immune escape. Nat Rev Microbiol 19:409-424. DOI: 10.1038/s41579-021-00573-0
22 Source: https://www.riziv.fgov.be/nl/riziv
23 Summary of Product Characteristics, December 2023, (the link can be accessed at info@pharma.be)
24 Hammond, J., H. Leister-Tebbe, A. Gardner, P. Abreu, W. Bao, W. Wisemandle, M. Baniecki, V. M. Hendrick, B. Damle, A. Simon-Campos, R. Pypstra, J. M. Rusnak and E.-H. Investigators (2022). "Oral Nirmatrelvir for High-Risk, Nonhospitalized Adults with Covid-19." N Engl J Med 386(15): 1397-1408
25 Aggarwal, N.R., et al., Real-world use of nirmatrelvir–ritonavir in outpatients with COVID-19 during the era of omicron variants including BA. 4 and BA. 5 in Colorado, USA: a retrospective cohort study. The Lancet Infectious Diseases, 2023
26 Lewnard JA, et al. 2023. Effectiveness of nirmatrelvir-ritonavir against hospital admission or death: a cohort study in a large US healthcare system. Lancet Infect Dis 2023 https://doi.org/10.1016/ S1473-3099(23)00118-4
27 Schwartz, K. L., Wang, J., Tadrous, M., Langford, B. J., Daneman, N., Leung, V., Gomes, T., Friedman, L., Daley, P., & Brown, K. A. (2023). Population-based evaluation of the effectiveness of nirmatrelvir-ritonavir for reducing hospital admissions and mortality from COVID-19. CMAJ : Canadian Medical Association journal = journal de l'Association medicale canadienne, 195(6), E220–E226. https://doi.org/10.1503/cmaj.221608
28 Shah MM, Joyce B, Plumb ID, Sahakian S, Feldstein LR, Barkley E, Paccione M, Deckert J, Sandmann D, Gerhart JL, Hagen MB. Paxlovid associated with decreased hospitalization rate among adults with COVID-19 - United States, April-September 2022. Am J Transplant. 2023 Jan;23(1):150-155. doi: 10.1016/j.ajt.2022.12.004. Epub 2023 Jan 11. PMID: 36695616; PMCID: PMC9833372
29 Globocan 2020. Europe Fact Sheets. Last consulted on June 2022
30 Ahn S, et al. J Pathol Transl Med. 2020;54(1):34-44
31 Barok M, et al. Breast Cancer Res. 2014;16(2):209
32 Newsletter Transplant 2015-2021. Available at https://freepub.edqm.eu/publications. Last consulted on February 2024
33 Eurostam Report (A Europe-wide strategy to enhance transplantation of highly sensitized patients on the basis of acceptable HLA mismatches.) Available at https://cordis.europa.eu/project/id/305385/reporting. Last consulted on March 2024
34 Mamode N, et al. European Guideline for the Management of Kidney Transplant Patients With HLA Antibodies: By the European Society for Organ Transplantation Working Group. Transpl Int. 2022 Aug 10;35:10511. Available at https://pubmed.ncbi.nlm.nih.gov/36033645/. Last consulted on March 2024
35 Alelign T, Ahmed MM, Bobosha K, Tadesse Y, Howe R, Petros B. Kidney Transplantation: The Challenge of Human Leukocyte Antigen and Its Therapeutic Strategies. J Immunol Res. 2018 Mar 5;2018:5986740. Available at https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5859822/. Last consulted on March 2024
36 Heidt S, et al. Highly Sensitized Patients are Well Serves by Receiving a Compatible Organ Offer Based on Acceptable Mismatches. Front Immunol. 2021;12:687254. Available at https://pubmed.ncbi.nlm.nih.gov/34248971/. Last consulted on March 2024
37 Lonze BE, et al. IdeS (Imlifidase): A Novel Agent That Cleaves Human IgG and Permits Successful Kidney Transplantation Across High-strength Donor-specific Antibody. Ann Surg. 2018 Sep;268(3):488-496. doi

38 Canaud B, Kooman JP, Selby NM, Taal MW, Francis S, Maierhofer A, Kopperschmidt P, Collins A, Kotanko P. Dialysis-Induced Cardiovascular and Multiorgan Morbidity. Kidney Int Rep. 2020 Sep 9;5(11):1856-1869. Available at https://pubmed.ncbi.nlm.nih.gov/33163709/. Last consulted on March 2024
39 Redfield RR, et al. The mode of sensitization and its influence on allograft outcomes in highly sensitized kidney transplant recipients. Nephrol Dial Transplant. 2016 Oct;31(10):1746-53. doi: 10.1093/ndt/gfw099. Epub 2016 Jul 6. PMID: 27387475 Last consulted on March 2024
40 Furian et al. Managing Sensitized Kidney Recipients.Transplant International, Frontiers 9 april 20254, Volume 37, Article 12475
41 S. Jordan, et al. Long-Term Follow Up of Imlifidase Desensitized Kidney Transplant Recipients: 5 Year Pooled Analysis. Presented at the American Transplant Congress, June 2024
42 Poggio ED, et al. Long-term kidney transplant graft survival-Making progress when most needed. Am J Transplant. 2021 Aug;21(8):2824-2832. doi: 10.1111/ajt.16463. Last consulted on February 2024
43 Kankerregister 2021
44 Palumbo A, A.-L. H. (2015). Revised International Staging System for Multiple Myeloma: A report from International Myeloma Working Group. Journal of clinical oncology: official journal of the American Society of Clinical Oncology, 33(26):2863-286
45 Delforge, M., Vekemans, M.-C., Anguille, S., Depaus, J., Meuleman, N., Van de Velde, A.,Vandervennet, S. (2021). Real-World Outcomes for Standard-of-Care Treatments in Patients with Relapsed/Refractory Multiple Myeloma. Blood 2021; 6(12):e813
46 Moreau, P. G.-M. (2022). Teclistamab in Relapsed or Refractory Multiple Myeloma. New England Journal of Medicine, 387 (6):495-505
47 Johnsen AT, T. D. (2009). Health related quality of life in a nationally representative sample of haematological patients. European Journal of Haematology, 83, 139-148
48 Mateos MV, N. H. (2020). Subcutaneous versus intravenous in patients with relapsed or refractory multiple myeloma (COLUMBA): a multicenter, open-label, non-inferiority, randomised, phase 3 trial. The Lancet, published online on 23 March
49 R.J. Motzer, T.K. Choueiri, T. Hutson et al., Characterization of Responses to Lenvatinib plus Pembrolizumab in Patients with Advanced Renal Cell Carcinoma at the Final Prespecified Survival Analysis of the Phase 3 CLEAR Study, Eur Urol (2024), https://doi.org/10.1016/j.eururo.2024.03.015
50 Albiges L, Gurney H, Atduev V, Suarez C, Climent MA, Pook D, Tomczak P, Barthelemy P, Lee JL, Stus V, Ferguson T, Wiechno P, Gokmen E, Lacombe L, Gedye C, Perini RF, Sharma M, Peng X, Lee CH. Pembrolizumab plus lenvatinib as first-line therapy for advanced non-clear-cell renal cell carcinoma (KEYNOTE-B61): a single-arm, multicentre, phase 2 trial. Lancet Oncol. 2023 Aug;24(8):881-891. doi: 10.1016/S1470-2045(23)00276-0. Epub 11 July 2023. PMID: 37451291
51 EAU Guidelines. Edn. Presented at the EAU Annual Congress, Paris 2024 ISBN 978-94-92671-23-3
52 Kankerregister 2019
53 Foulkes WD, Smith IE, Reis-Filho JS. Triple-negative breast cancer. N Engl J Med. 2010;363(20):1938-48
54 Sharma P. Biology and Management of patients with triple-Negative Breast Cancer. Oncologist. 2016;21(9):1050-62
55 American Cancer Society. https://www.cancer.org/cancer/types/breast-cancer/risk-and-prevention/breast-cancer-risk-factors-you-cannot-change.html. Consulted on 31 May 2022
56 Linda Lindstrom et al. Clinically used breast cancer markers such as estrogen receptor, progesterone receptor, and human epidermal growth factor 2 are unstable throughout tumor progression. J of Clin Oncol 2012;30(21):2601-8
57 Lee A, Djamgoz MBA. Triple negative breast cancer : Emerging therapeutic modalities and novel combination therapies. Cancer Treat Rev. 2018;62:110-22
58 Shah MV et al. Patient-reported symptoms and impacts of locally advanced or metastatic urothelial cancer (la/mUC) after chemotherapy followed by a PD-1/PD-L1 checkpoint inhibitor (CPI). J Clin Oncol 2019; 37 (7_suppl). Poster presented on ASCO GU, 14-16 februari 2019. Abstract 380
59 National Cancer Institute 2023. Bladder Cancer Symptoms. Available at https://www.cancer.gov/types/bladder/symptoms. Last consulted on April 2024
60 Kankerregister 2022. Cancer Burden in Belgium 2004-2019. Available at www.kankerregister.org. Last consulted on April 2024
61 Maiorano et al. Enfortumab vedotin in metastatic urothelial carcinoma: the solution EVentually? Front Oncol 2023; 13:1254906
62 Powles T et al. Enfortumab Vedotin in Previously Treated Advanced Urothelial Carcinoma. N Engl J Med 2021; 384:1125-1135
63 Rosenberg J et al. Health-related Quality of Life in Patients with Previously Treated Advanced Urothelial Carcinoma from EV-302: A Phase 3 Trial of Enfortumab Vedotin Versus Chemotherapy. Eur Urol 2024
64 Summary of Product Characteristics, 2021 (the link can be accessed at info@pharma.be)
65 Damato BE, Dukes J, Goodall H, Carvajal RD. Tebentafusp: T Cell Redirection for the Treatment of Metastatic Uveal Melanoma. Cancers (Basel). 11 juli 2019;11(7):971. doi: 10.3390/cancers11070971. PMID: 31336704; PMCID: PMC6679206
66 Nathan, P.; Hassel, J.C.; Rutkowski, P.; Baurain, J.F.; Butler, M.O.; Schlaak, M.; Sullivan, R.J.; Ochsenreither, S.; Dummer, R.; Kirkwood, J.M.; et al. Overall Survival Benefit with Tebentafusp in Metastatic Uveal Melanoma. N. Engl. J. Med. 2021, 385, 1196–1206
67 Hassel JC, Piperno-Neumann S, Rutkowski P, Baurain JF, Schlaak M, Butler MO, Sullivan RJ, Dummer R, Kirkwood JM, Orloff M, Sacco JJ, Ochsenreither S, Joshua AM, Gastaud L, Curti B, Piulats JM, Salama AKS, Shoushtari AN, Demidov L, Milhem M, Chmielowski B, Kim KB, Carvajal RD, Hamid O, Collins L, Ranade K, Holland C, Pfeiffer C, Nathan P. Three-Year Overall Survival with Tebentafusp in Metastatic Uveal Melanoma. N Engl J Med. 14 December 2023;389(24):2256-2266. doi: 10.1056/NEJMoa2304753. Epub 21 October 2023. PMID: 37870955
68 Belgium, the Czech Republic, Denmark, Finland, France, Germany, Greece, Italy, Lithuania, the Netherlands, Norway, Romania, Spain, Slovenia, Sweden, Turkey and the United Kingdom
69 Based on an interview with Stéphane Petit (Chairman of the Board, BeCRO) and Piet Smet (Vice Chairman of the Board, BeCRO)
70 Source: https://www.famhp.be/en/human_use/medicines/medicines/research_development/compassionate_use_medical_need
71 https://circabc.europa.eu/ui/group/8ee3c69a-bccb-4f22-89ca-277e35de7c63/library/051e5787-7746-46cf-8a0d-310f84fd1900/details?download=true
72 Source: https://www.rarediseasemoonshot.eu/

Acknowledgements

This Report to Society would not have been possible without the contributions of pharma.be colleagues who provided content, pharma.be members who described their newly reimbursed medicines, BeCRO for their section on collaboration with Contract Research Organisations, PwC Belgium for their input on the ESG chapter, the stakeholders who provided quotes, Conny Van Gheluwe from Sproke for copywriting, and Karakters for the layout of this report.

VZW/ASBL (NPO) – BE 0407.622.902 – RLE Brussels – Legal deposit: D/2024/4021/03
Responsible publisher: Caroline Ven – Kantersteen 47 Cantersteen, 1000 Brussels, Belgium
www.pharma.be – info@pharma.be
VAT BE 0407.622.902 – Company registration number 0407.622.902

pharma.be

ASSOCIATION GÉNÉRALE DE L'INDUSTRIE DU MÉDICAMENT
ALGEMENE VERENIGING VAN DE GENEESMIDDELENINDUSTRIE

pharma.be asbl/vzw
Kantersteen 47 Cantersteen,
1000 Brussels – 02 661 91 11
info@pharma.be – www.pharma .be