Business Report

idorsia

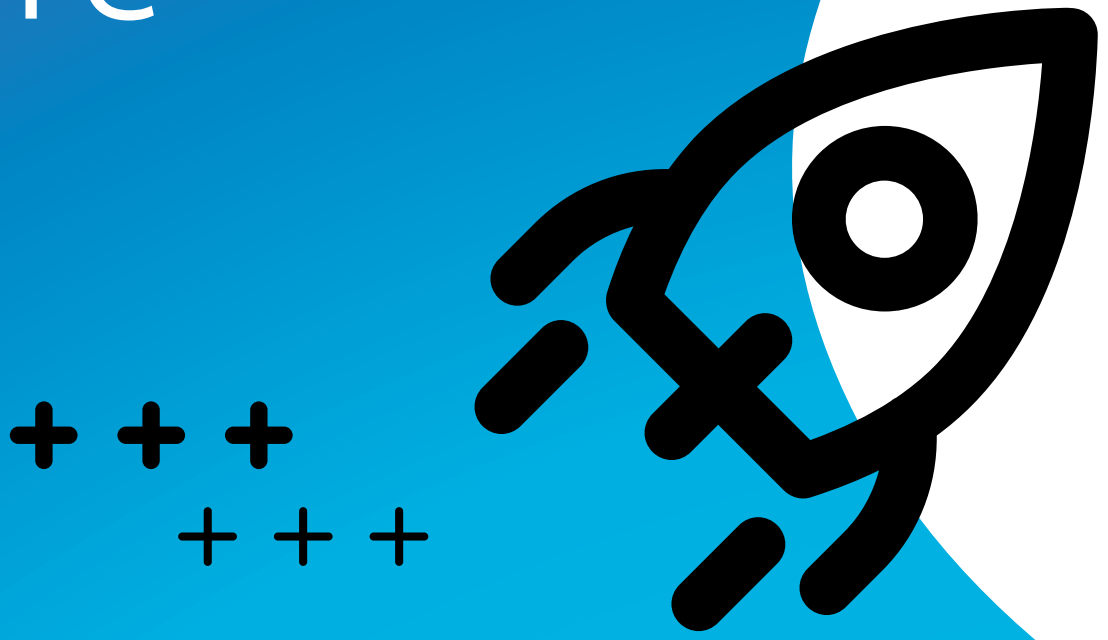
15 June 2017

Power

The purpose of Idorsia is to discover, develop and bring more, innovative medicines to patients.

We have more ideas, we see more opportunities and we want to help more patients.

Idorsia – Reaching out for more



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> 650

Highly qualified professionals

11

Compounds in the pipeline, with four progressing to late-stage development in the year ahead

>1 bn

Swiss francs in cash

> 550

State-of-the-art laboratory workspaces

More science – Bursting with ideas

Idorsia began its operations after the demerger from Actelion on June 15, 2017, and registered shares of Idorsia Ltd were listed on the SIX Swiss Exchange the following day. Today, Idorsia has an experienced team of highly qualified professionals, a full R&D pipeline, state of-the-art facilities, and more than CHF 1 billion in cash – the ideal constellation for bringing successful medicines to the market.

Idorsia is so much more than a start-up!

Idorsia's key numbers	**Period ended December 31, 2017**	
in CHF millions (except EPS)	**US GAAP**	**Non-GAAP**
Revenues	158	158
Operating expenses	(166)	(150)
Operating income (loss)	(8)	8
Net income (loss)	(14)	5
Basic EPS	(0.13)	0.04
Basic number of shares (weighted average)	114.0	114.0
Diluted EPS	(0.13)	0.03
Diluted number of shares (weighted average)	114.0	139.5

The full financial statements can be found in the Financial Report which forms part of the Idorsia Annual Report.

How Idorsia was born

3 2 1

There is usually not much debate about the exact date of a child's birthday; however, when it comes to the birthday of a company like Idorsia, one could argue:

Was it January 26, 2017, when Actelion first announced that – as part of the transaction with Johnson & Johnson – it would spin out its drug discovery operations and early-stage clinical development assets into a newly created Swiss biopharmaceutical company? Or was it March 2, the day of incorporation, when Idorsia gained its own legal business structure? Or June 15, the day when its shares were listed and trading commenced on the SIX Swiss Exchange?

Whatever your opinion may be about the company's actual birthday, there is something we at Idorsia can all agree on: this "baby" was born thanks to the energy and can-do mindset of everyone involved. In building the new company and separating its day-to-day activities from those of its next door neighbor Actelion, it soon became clear that determination would be required to make Idorsia a reality. The challenges were manifold and by no means only of a scientific nature. For example, 40,000

June 16, 2017: CEO Jean-Paul Clozel and CFO André C. Muller ringing in the first trading day for Idorsia at the SIX Swiss Exchange in Zurich.

contracts had to be transferred. Who was to pay the electricity and water bills for which buildings? Were there separate meters? What about access to intellectual property, scientific databases, IT infrastructure, and – last but certainly not least – the canteen?

Resolving issues like these in a short timeframe required not only an atmosphere of mutual trust, but also pragmatism and a sense of priorities. Today, about a year after the proposed creation of Idorsia was first announced, it can rightly be said that all parties played their full part in the months that followed, and that their constructive approach has been rewarded: Idorsia has been fully functional since the demerger from Actelion, and any remaining separation activities continue well on track, making this innovative transaction a great success.

With the rich collection of compounds brought over from Actelion, the Idorsia teams are now working hard to advance the company's highly innovative pipeline and further enhance the value proposition from these assets. Discussions with health authorities are progressing well, so that key compounds are expected to move into late-stage development in the coming year.

Just as in bringing up a child, intelligent decision-making will be crucial if Idorsia is to continue to grow and thrive in the short and long term. Science and innovation need to be put at the center of these decisions – to translate our ideas into something tangible that will not only add value for investors but will benefit patients, who should always be the ultimate concern.

"Having invested so much effort in building a research engine and a diversified pipeline at Actelion, I am delighted that we can continue to work on these important projects at Idorsia, to realize their potential and advance many fields of medicine."

Jean-Paul Clozel
CEO

I was there on the first day of Idorsia Ltd – this is the inscription on the founding stone received as a memento by all Idorsia employees.

Dear Shareholders

"Idorsia is off to a very positive start with impressive clinical results and productive discussions with health authorities. We have made great progress to establish the company while advancing our pipeline without any loss of momentum."

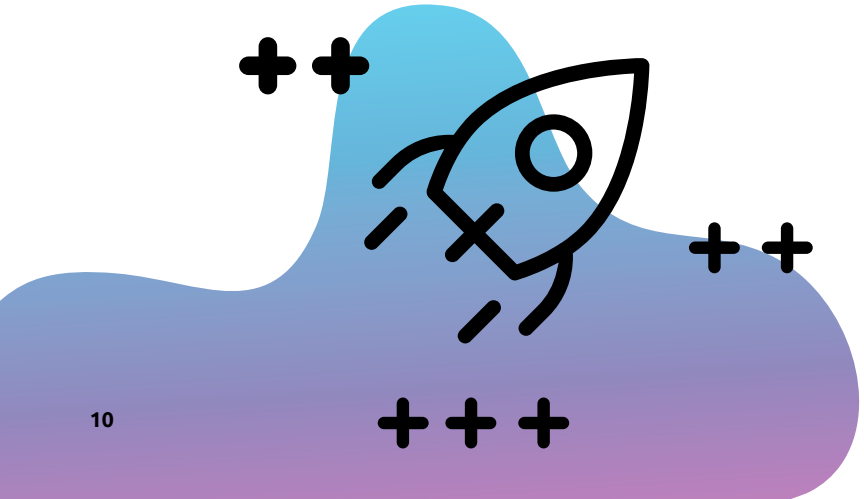
Jean-Paul Clozel
CEO

2017 has been an exciting year! We started off by announcing our intention to create a new biopharmaceutical company in January, the company was listed in June, and now, just a few months later, Idorsia is in full motion. The innovative transaction with Johnson & Johnson allowed Actelion's shareholders to monetize their holdings at a highly attractive cash price of USD 280 per share, while at the same time – through ownership of Idorsia – retaining a significant stake in the potential upside of Actelion's early stage pipeline.

Getting to this point has required a lot of work, and there is a still a lot to be done, but we have every reason to be optimistic about our future. We are starting off with four key strengths necessary for success. We have a rich and innovative R&D pipeline, more than 650 skilled employees, state-of-the-art equipment and laboratories, and more than 1 billion Swiss francs in cash.

Building a sustainable company

We have set our sights on becoming one of Europe's leading, fully fledged biopharmaceutical companies. Based in Allschwil, we are focused on discovering, developing, delivering and distributing innovative medicines. We intend to invest judiciously in our pipeline, taking a structured approach to spending, with the goal of building a profitable and sustainable company. By the end of 2018, we expect to have largely completed the process of separation from Actelion. More importantly, we are very proud that – despite the significant changes that our teams have had to cope with – our innovative pipeline projects have advanced and remain on track.



Advancing our assets to the next level

In 2017, we concluded several Phase 2 studies and have been able to engage with the regulatory authorities to further advance these compounds. In the course of 2018, we aim to move four of these projects into Phase 3 – a crucial step in reaching our key strategic priority of launching at least three innovative products and attaining profitability and financial sustainability within the next 5 years.

Flexibility through partnerships

One way of enhancing the value proposition of our pipeline is to seek partnerships for certain assets where we believe we can benefit from sharing development costs – as well as the inherent risks – and integrating expertise and capacity from other successful teams. Of course, if we choose this path, we will also be sharing the rewards, so we need to be very careful when deciding which assets to share.

Johnson & Johnson, recognizing the huge potential of one of our assets, have teamed up with us in the development of aprocitentan, thus becoming not only a significant shareholder but also a partner. Together, we can realize the full potential of aprocitentan, first in resistant hypertension, and then – thanks to our partner's broad expertise in cardiovascular indications and global footprint – unlocking significant value in additional indications.

"Idorsia is able to fully leverage the excellent R&D pipeline that was created at Actelion. The Board has great confidence in the team and I am convinced that their tremendous track record is going to continue to translate into value creation for all."

Jean-Pierre Garnier
Chairman

"Idorsia really has potential to transform science into meaningful therapeutic options for many patients."

Jean-Paul Clozel
CEO

Managing risk and opportunities

The fact that we are in such a strong position after less than a year of operations demonstrates that Idorsia is a start-up like no other. The company already has sound governance structures in place: as members of management and the Board have worked together before, a framework has been rapidly and efficiently established to ensure that Idorsia has the right strategy to create value for shareholders. Because we also have a thorough understanding of the risks associated with Idorsia's business – based on our experience in the industry and an intimate knowledge of our assets – we are in an excellent position to minimize risk and maximize value creation

Doing more with less

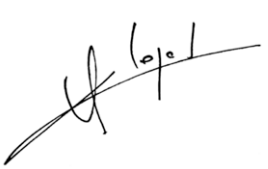
As investment will be essential in the coming years, we are fortunate to be starting out with 1 billion in cash, and our business development activities have led to additional cash injections. However, until we are generating our own revenue and are truly sustainable, we must be highly cost conscious. This message is constantly repeated at all levels of Idorsia, and our teams are well aware that they are expected to achieve ambitious targets, to work in an ethical manner, and to keep a keen eye on the bottom line.

Bearing this in mind, with our current plans, we expect our non-GAAP operating expenses for 2018 to be around 390 million Swiss francs, mainly depending on when each of the different Phase 3 programs commences.

Creating significant value

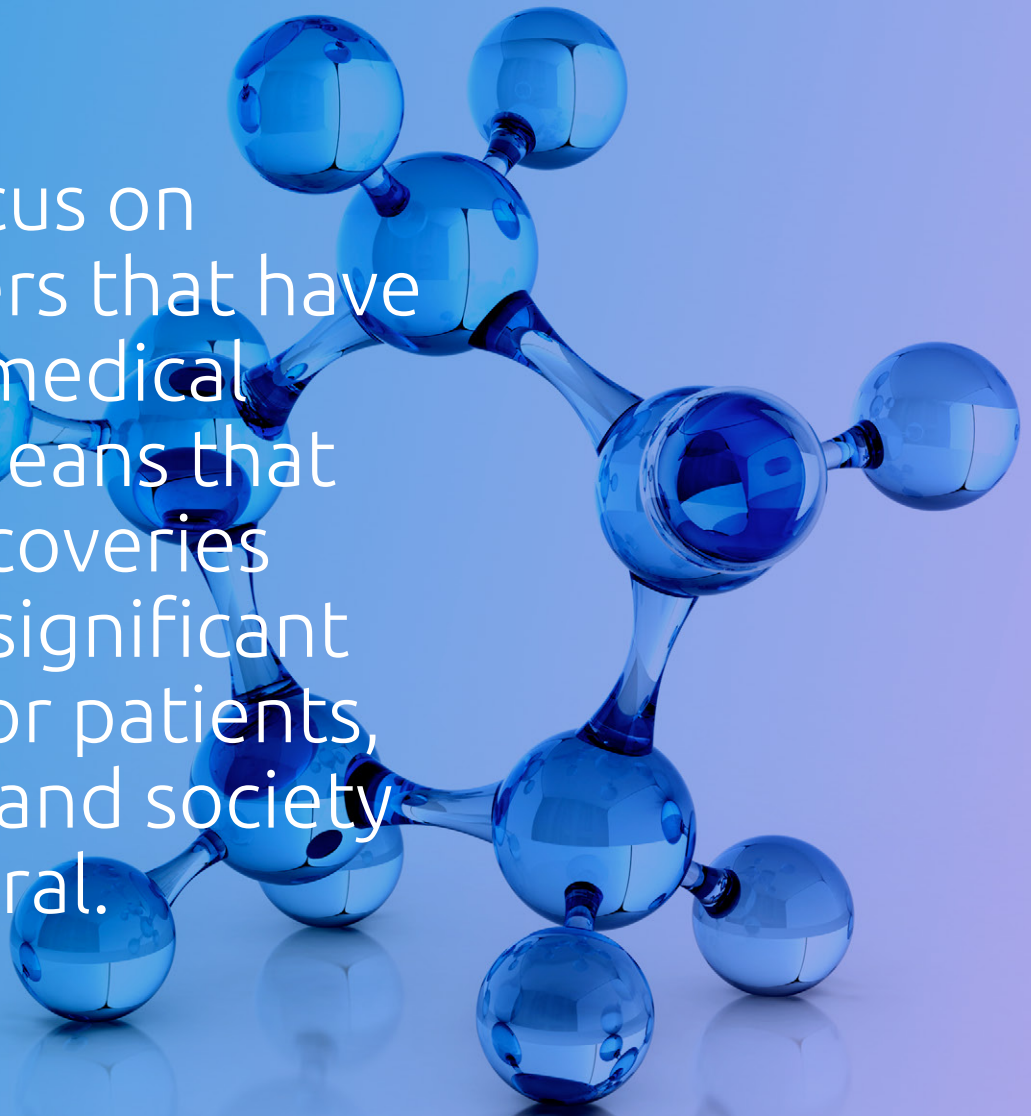
A huge amount has been achieved in 2017. The task ahead of us is to repeat and even exceed our previous success by rapidly transforming Idorsia from a clinical-stage biopharmaceutical company into one with innovative products on the market, changing the way patients are treated. We have identified our key strengths, defined an ambitious strategy, advanced our pipeline and initiated the interaction with partners. We hope you will continue to give us your confidence as we **reach out for more**.

Sincerely,



Jean-Paul Clozel
CEO

Jean-Pierre Garnier
Chairman



Our focus on disorders that have a high medical need means that our discoveries create significant value for patients, payors and society in general.

Our Research & Development

11 **compounds in our clinical development pipeline**

More knowledge – Powered by science

At Idorsia, our drug discovery approach focuses on families of proteins, which are characterized by the way they work. We call this the “platform approach”.

We strive to identify innovative programs on proteins which have not been targeted up to now, to discover drugs with novel mechanisms of action.

The drug discovery process starts with an idea from our scientists. We scour the literature to see what others have not yet discovered, to generate ideas and then translate them into a concept which can lead to new treatments for patients.

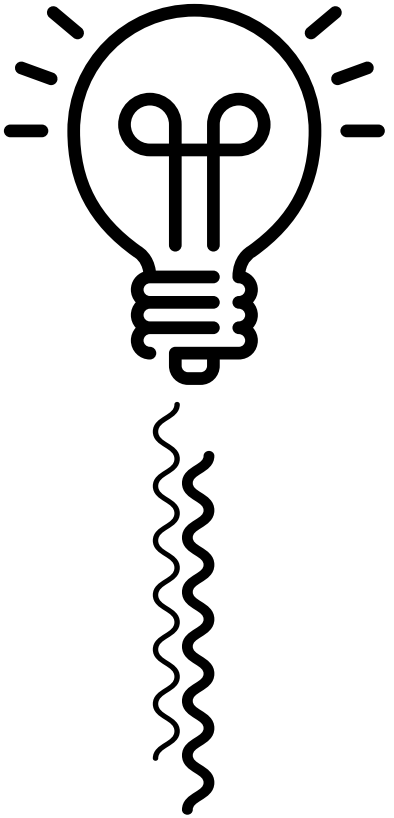
Our work in the lab begins with the target. This may be a particular protein which, when its activity is modulated, can normalize a biological process in the body – with a beneficial effect for patients. To see whether we can affect the protein’s activity, we first need to be able to measure it.

We produce, or “express”, the target in large quantities and measure its natural activity in assays. The assay needs to be sensitive, accurate and highly reliable. Plus, in order to perform hundreds of thousands of measurements, it needs to be automated, using robotic equipment.

But there are two sides to the discovery process – a target and a compound.

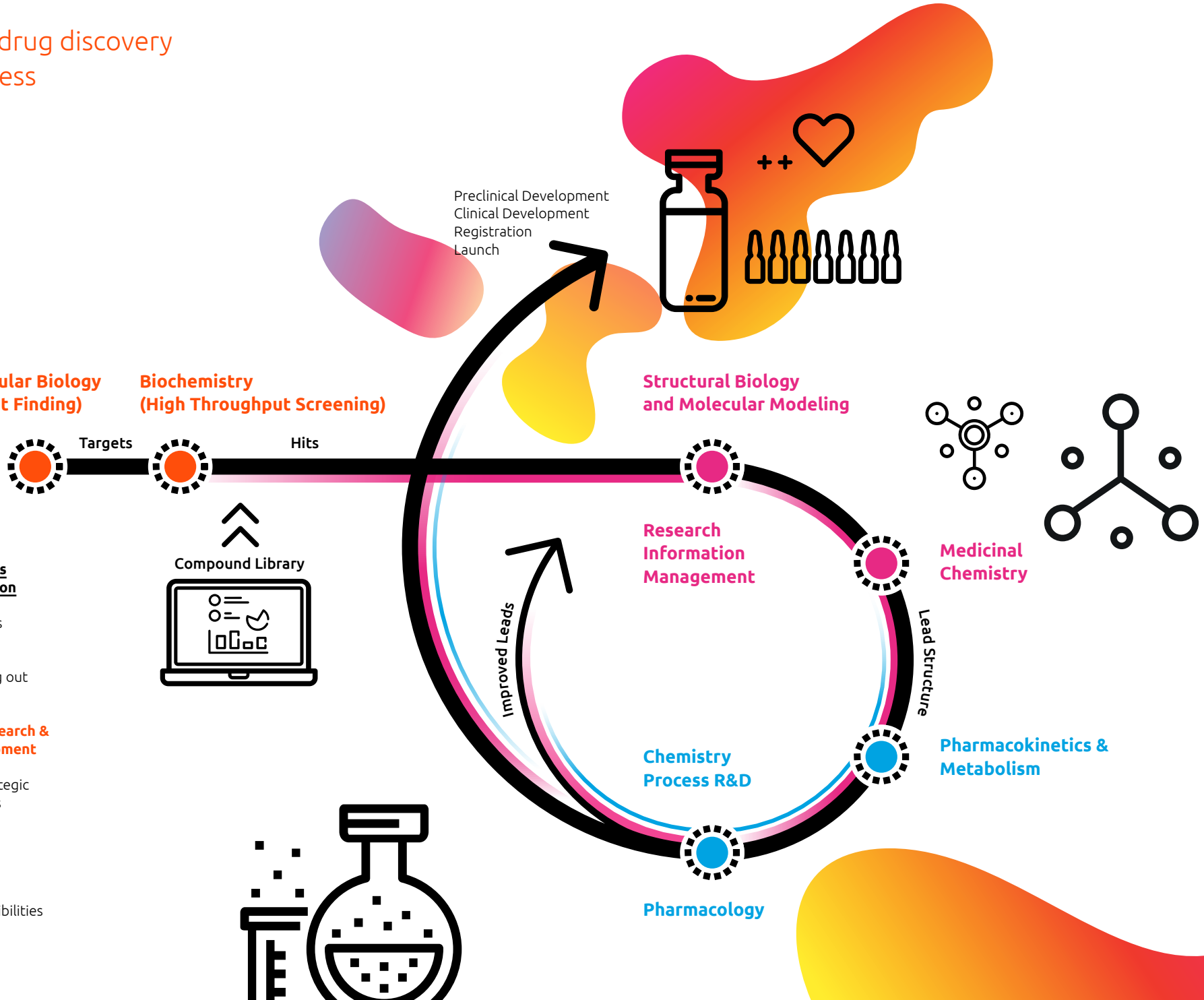
Compounds are substances which, we hope, will modify the activity of a target involved in a pathological process and can then be developed into a drug for patients.

At Idorsia, we maintain a library consisting of hundreds of thousands of different compounds. To begin our hunt for drugs, we test the entire library of compounds on the target, in the hope that one of them will modify the activity of the protein. This process is called high throughput screening; if it’s a simple assay, we can test the whole library within a matter of weeks. At this stage, the goal is to identify compounds which exhibit some activity.



“We need creativity to be innovative, so we need a brilliant idea and a deep understanding of the disease, to translate it into a molecular mechanism, and to try to find a drug to treat that disease.”

Our drug discovery process



The project team then analyzes these compounds to decide which of them is the most promising starting point for optimization using the art of medicinal chemistry.

Obviously, huge amounts of data are generated, and powerful IT tools are required to extract the knowledge we need. To really understand the data, we visualize them and study the relationship between chemical structures and biological properties.

Target and compound fit together like a lock and a key. The compound can be modified so that it fits better and, ideally, becomes more potent.

Medicinal chemistry involves the use of chemistry's tools to design molecules that are potential drugs. We manipulate the molecular structure and then send the compounds back to our biologists or pharmacologists for testing in an iterative process. With each cycle the compound is further optimized to finally become a drug.

At first, we seek to enhance the potency of its effects on the target protein, but as we advance we look at other activities which may cause side effects. The aim is to ensure that the compound's overall properties allow it to become a drug.

For example, our electrophysiologists screen drugs for side effects by monitoring electrical activity in the heart or brain. Here, electrical communication depends on ion channels in the cell membrane; if a drug blocks some of these ion channels, it can have serious adverse effects.

Small-scale testing for initial assays requires only milligram quantities; for subsequent testing, however, much more material is needed. This is where our process research teams come into the picture. They are responsible for scaling up from milligram to gram quantities, and finally to the kilogram batch which is used for preclinical testing.

It's no good having a potent compound which gets destroyed by the body before it has a chance to do its job. Our formulation specialists take a compound which has been optimized by the chemists and ask how it can best be delivered to the patient. One way to protect the compound, for example, is to package it in a capsule; alternatively, it may be better to develop an injectable form.

For Idorsia, the process which begins with drug discovery and preclinical development ends, we hope, with a novel molecule that will help patients in diseases which still have a high medical need.

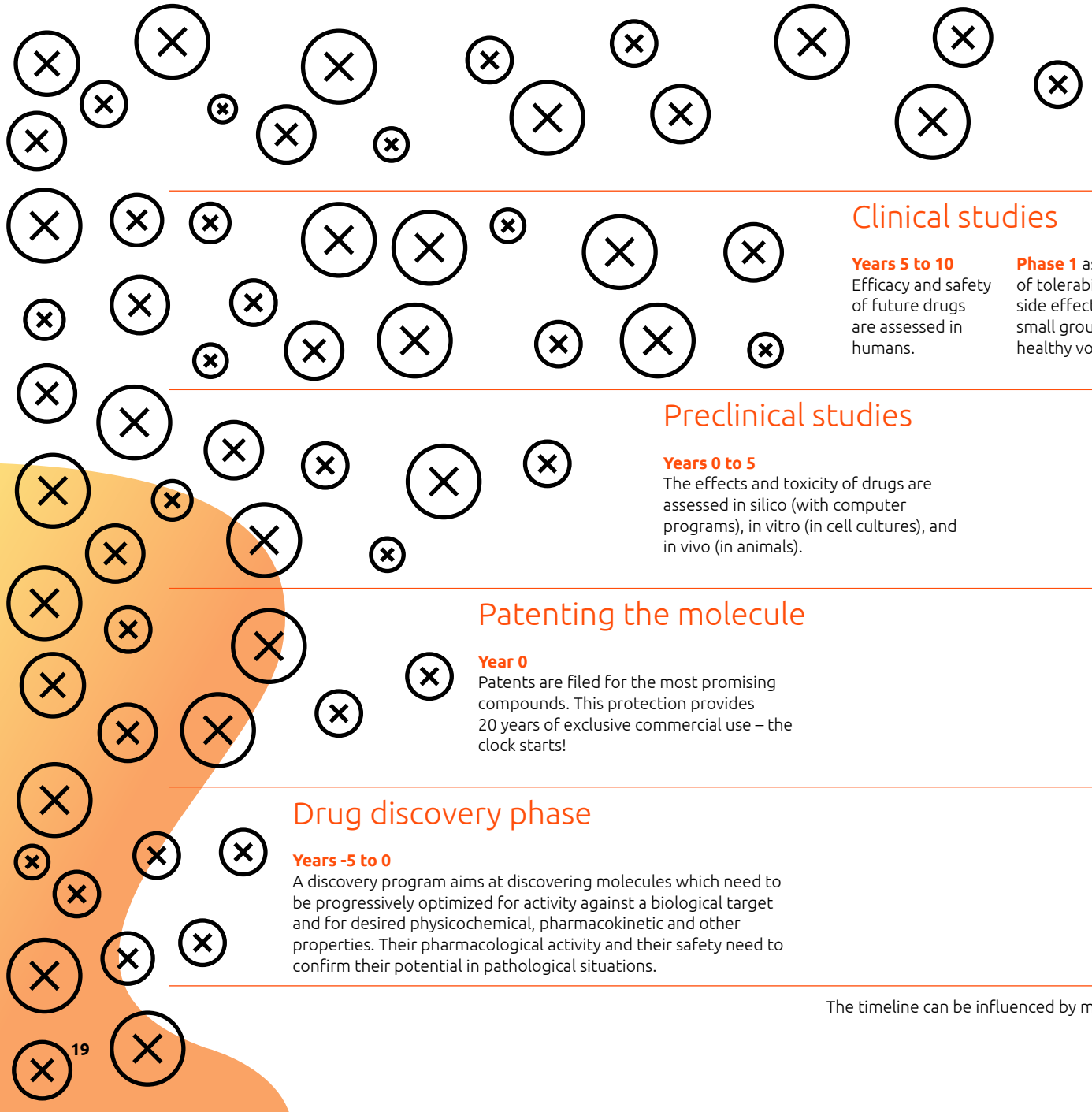
"People are highly motivated and individual ideas can affect the whole process."

"For me, invention is making something out of a daring idea. And I really have the feeling that's what we are trying to do at Idorsia."

"This is a group of extraordinary, highly dedicated people, and over the next few years we may see the fruits of all their work – which has the potential to change the lives of so many patients."

More molecules – For a better future

Following the drug discovery phase, the selected molecule must be comprehensively studied to demonstrate clinical safety and efficacy.



Regulatory submission

Years 10 to 12
Before a drug can be placed on the market, it must first be approved by local regulatory authorities. A comprehensive dossier is submitted for approval.

Clinical studies

Years 5 to 10
Efficacy and safety of future drugs are assessed in humans.

Phase 1 assessment of tolerability or side effects in a small group of healthy volunteers.

Phase 2 assessment of the safety and efficacy of the compound in a limited number of patients, with the aim of finding the optimal dose for large-scale studies.

Phase 3 assessment of the safety and efficacy of a future medicine compared to placebo in a large group of patients.

Preclinical studies

Years 0 to 5
The effects and toxicity of drugs are assessed in silico (with computer programs), in vitro (in cell cultures), and in vivo (in animals).

Patenting the molecule

Year 0
Patents are filed for the most promising compounds. This protection provides 20 years of exclusive commercial use – the clock starts!

Drug discovery phase

Years -5 to 0
A discovery program aims at discovering molecules which need to be progressively optimized for activity against a biological target and for desired physicochemical, pharmacokinetic and other properties. Their pharmacological activity and their safety need to confirm their potential in pathological situations.

The timeline can be influenced by many factors, such as the indication for which a drug is being studied.

More in the pipeline – Promising compounds

We have a diversified and balanced clinical development pipeline covering multiple therapeutic areas, including CNS, cardiovascular and immunological disorders, as well as orphan diseases.

We bring new perspectives to the development of innovative compounds, challenging accepted paradigms to answer the questions that matter to patients. Our key assets have the potential to transform treatment in the target indications.

Clinical Development Pipeline

Resistant hypertension management

Aprocitentan
Dual endothelin receptor antagonist
Status: Advancing to Phase 3

Insomnia

ACT-541468
Dual orexin receptor antagonist
Status: Advancing to Phase 3

Fabry disease

Lucerastat
Glucosylceramide synthase (GCS) inhibitor
Status: Advancing to Phase 3

Vasospasm associated with aneurysmal subarachnoid hemorrhage (aSAH)

Clazosentan
Endothelin receptor antagonist
Status: Advancing to Phase 3

Systemic lupus erythematosus

Cenerimod
$S1P_1$ receptor modulator
Status: Phase 2

Duchenne muscular dystrophy

Vamorolone
Dissociative steroid
Status: Phase 2
Idorsia has an exclusive option to the worldwide rights for ReveraGen's vamorolone

Acute Coronary Syndrome

ACT-246475
P2Y12 receptor antagonist
Status: Phase 2

Orphan CNS diseases

ACT-519276
GBA2/GCS inhibitor
Status: Phase 1

Asthma and allergy disorders

ACT-774312
CRTH2 receptor antagonist
Status: Phase 1

Anxiety

ACT-539313
Selective orexin 1 receptor antagonist
Status: Phase 1

Epilepsy

ACT-709478
T-type calcium channel blocker
Status: Phase 1

More hope – Pioneering therapies

With a promising clinical development pipeline, Idorsia is well positioned to develop new and differentiated products in multiple therapeutic areas.

Lucerastat for Fabry disease

Lucerastat is an oral monotherapy offering a new treatment approach with the potential to alleviate the symptoms of Fabry disease.

Fabry disease is a rare genetic disorder involving a deficiency or dysfunction of α galactosidase A – an enzyme that normally breaks down a fatty waste product known as Gb3 in the cells of the body. Over time, this may result in a build-up of Gb3 deposits throughout the body, particularly in the kidneys, heart and nervous system. The symptoms range from pain in the hands and feet, eye and stomach problems, to stroke and kidney failure, depending on which organs are affected. Because the symptoms are non specific, Fabry disease is often undetected or misdiagnosed. As the disease is progressive, early diagnosis is essential to manage the symptoms as soon as possible and reduce the risk of developing serious complications.

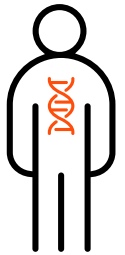
Current treatment approaches for Fabry disease include enzyme replacement therapy (ERT), which requires frequent intravenous infusions to replace the deficient enzyme in the cells with a genetically engineered form.

Lucerastat is a small-molecule iminosugar which inhibits glucosylceramide synthase and has the potential to provide substrate reduction therapy for oral treatment of Fabry disease.

In an exploratory study in patients with Fabry disease, treatment with lucerastat in addition to ERT demonstrated a marked decrease in plasma levels of metabolic substrates associated with the disease. The study also demonstrated that lucerastat is well tolerated in patients with Fabry disease.

In the first half of 2018, Idorsia expects to initiate a pivotal Phase 3 study designed to assess the effects of lucerastat on neuropathic pain and gastrointestinal symptoms, as well as safety and tolerability, in patients with Fabry disease. The study is expected to enroll around 100 patients and to last approximately 20 months.

Fabry disease
Compound: Lucerastat



Mechanism of action:
Glucosylceramide synthase inhibition
Status: Advancing to Phase 3

Lucerastat for Fabry disease has received Orphan Drug designation in the US and in the EU and at the beginning of 2018, the European Medicines Agency (EMA) agreed with Idorsia's paediatric investigation plan for lucerastat for the treatment of pediatric patients with Fabry disease. Idorsia has already initiated activities according to the agreed plan.

Aprocitentan for resistant hypertension management

Aprocitentan is an orally active dual endothelin receptor antagonist, which is being investigated for patients whose hypertension is uncontrolled despite the use of three or more antihypertensive drugs.

Resistant hypertension is associated with a higher risk of cardiovascular disease. In addition, patients with resistant hypertension often have a medical history of diabetes mellitus and may develop chronic kidney disease as a complication of the hypertension, increasing their vulnerability and the complexity of treatment.

The need for another mechanism of action in the treatment of patients with resistant hypertension has long been stressed by the medical community. Aprocitentan has great potential in the management of resistant hypertension, as an oral, once daily treatment with potent, long-lasting antihypertensive effects.

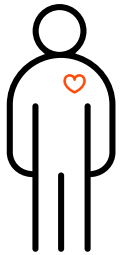
In a Phase 2 study – completed in May 2017 – the efficacy, safety and tolerability of aprocitentan was evaluated in patients with essential hypertension so as to identify the optimal dose for further studies.

Based on the positive results from the dose-finding study and following feedback from health authorities, Idorsia is currently finalizing the design of a Phase 3 study. This will be specifically designed to evaluate the initial and long-term effects of aprocitentan on systolic and diastolic blood pressure in patients requiring resistant hypertension management (RHM). The study is expected to start in the first half of 2018. If successful, it will provide the basis for registration of the product.

On December 1, 2017, Janssen Biotech, Inc. (one of the Janssen Pharmaceutical Companies of Johnson & Johnson) entered into a collaboration agreement with Idorsia to jointly develop and commercialize aprocitentan and any of its derivative compounds or products. Idorsia received a one-time milestone payment of USD 230 million. Both parties have joint development rights over aprocitentan. Idorsia will oversee Phase 3 development and regulatory submission for the first indication. The costs will be shared equally between the two partners. Janssen will oversee Phase 3 development and submission for any additional indications.

Resistant hypertension management
Compound: Aprocitentan

Mechanism of action:
Dual endothelin receptor antagonism
Status: Advancing to Phase 3



Dual orexin receptor antagonist for insomnia

ACT-541468 is a dual orexin receptor antagonist (DORA) for the treatment of insomnia. It has potential for fast onset of sleep and a duration of action not exceeding a normal night, while maintaining natural sleep architecture.

Insomnia – the most commonly reported sleep disorder worldwide – is defined as a combination of dissatisfaction with sleep and a significant negative impact on daytime functioning. Dissatisfaction with sleep refers to difficulty in initiating and/or maintaining sleep on at least three nights per week for at least three months, despite adequate opportunity to sleep.

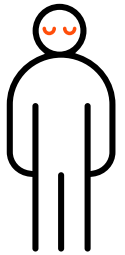
The impacts of insomnia on physical and mental health may include fatigue, daytime sleepiness, poor concentration, depressed mood, or impaired ability to perform social or occupational tasks. The goal of treatments for insomnia is to improve sleep quality and quantity, as well as reducing insomnia-related daytime impairments, while avoiding adverse effects and next-day residual effects.

ACT-541468 is a dual orexin receptor antagonist (DORA), targeting the orexin system. Orexins are small, protein-like molecules used by nerve cells to communicate with each other in the brain. They play an important role in the regulation of sleep and arousal. Orexin is an awakening peptide.

The safety and efficacy of ACT-541468 in adult and elderly patients with insomnia was evaluated in a comprehensive Phase 2 program, comprising two studies. Both studies showed the desired effect on sleep maintenance and onset, with a significant dose-response relationship; treatment was generally well tolerated.

In the first half of 2018, following feedback from health authorities, Idorsia expects to initiate a pivotal Phase 3 registration program. The program consists of three studies designed to evaluate time to sleep onset, sleep maintenance, and next day performance, as well as providing long-term safety information, in patients with insomnia.

Insomnia
Compound: ACT-541468



Mechanism of action:
Dual orexin receptor antagonism
Status: Advancing to Phase 3

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Clazosentan for cerebral vasospasm associated with aSAH

Clazosentan is an endothelin receptor antagonist being developed as an intravenous infusion for the prevention and treatment of cerebral vasospasm in patients who have suffered an aneurysmal subarachnoid hemorrhage (aSAH).

Aneurysmal subarachnoid hemorrhage (aSAH) is a sudden life-threatening bleeding occurring in the subarachnoid space (i.e., between two layers of the protective membranes surrounding the brain). It is caused by the rupture of an aneurysm – a weak, bulging spot on the wall of a brain artery – which allows blood to escape and accumulate in the space around the brain. Surgical repair (endovascular coiling or microsurgical clipping) is required to prevent fatal rebleeding.

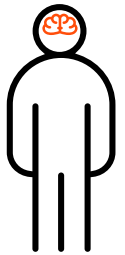
In about one third of patients with aSAH, worsening of the neurological condition may occur due to delayed cerebral vasospasm (constriction of arteries in the brain). Cerebral vasospasm diminishes blood flow to the brain and may lead to cerebral infarction and poor long-term outcomes.

Currently, invasive endoarterial intervention is used to treat cerebral vasospasm. This therapy is associated with medical risks and often requires repeated procedures. No effective medication is available for the prevention or treatment of cerebral vasospasm.

Several studies have built our understanding of the effects of clazosentan on cerebral vasospasm, indicating that it has the potential to prevent ischemic complications of cerebral vasoconstriction and to decrease the need for invasive endoarterial intervention.

In Japan, two registration studies evaluating the safety and efficacy of clazosentan in reducing vasospasm-related morbidity and mortality events after aneurysm-securing procedures are being conducted. Results are expected in the second half of 2018.

Cerebral vasospasm
Compound: Clazosentan



Mechanism of action:
Endothelin receptor antagonism
Status: Advancing to Phase 3

Later in 2018, Idorsia expects to initiate a Phase 3 study evaluating the safety and efficacy of clazosentan in an aSAH population enriched for the risk of cerebral vasospasm.

Cenerimod for systemic lupus erythematosus

Cenerimod is a potent, orally active, selective sphingosine-1-phosphate receptor 1 ($S1P_1$) modulator, which potentially offers a novel approach for systemic lupus erythematosus (SLE) – a disease with limited treatment options.

SLE is the most common form of lupus, an autoimmune disease which can affect the skin, joints, kidneys, brain and other organs. While the cause of SLE is not fully known, T and B lymphocytes are considered to play a major role in the disease.

SLE is more common in women than men and most often appears in people between the ages of 15 and 40. According to the Lupus Foundation of America, at least five million people worldwide have some form of lupus. The number of people with the condition could be even higher, as many cases remain undiagnosed.

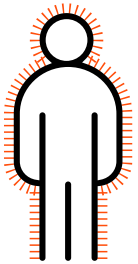
The course of the disease is characterized by relapses or flares, alternating with periods of remission. Symptoms vary from person to person, depending on the organ affected. Most common are joint pain and swelling, as well as skin conditions such as rash and sensitivity to light.

Cenerimod blocks the egress of lymphocytes from lymphoid organs, thus reducing the availability of circulating lymphocytes (T and B cells) that can invade target organs. This effect is sustained with continued daily oral dosing and is reversible upon drug discontinuation.

In a Phase 2 study in adult patients with SLE, cenerimod induced a dose-dependent reduction in lymphocyte count and was well tolerated at all dose levels.

In December 2017, the US FDA designated the investigation of cenerimod for the treatment of systemic lupus erythematosus as a Fast Track development program. The Fast Track designation is intended to promote communication and collaboration between the FDA and the company for drugs that treat serious conditions and fill an unmet medical need.

Systemic lupus erythematosus
Compound: Cenerimod



Mechanism of action:
$S1P_1$ receptor agonism
Status: Phase 2

Idorsia is currently discussing the development program with health authorities to advance cenerimod in this underserved disease as quickly as possible.

More drive – For more discoveries

It is less than a year since Idorsia started its journey as an independent biopharmaceutical company, and the team has already come a long way.

We caught up with Martine Clozel, Chief Scientific Officer, and Guy Braunstein, Head of Global Clinical Development, to get their perspective on how the journey has gone so far…

Let's kick off with your thoughts on establishing Idorsia and separating the activities from Actelion. What stands out for you as going particularly well – and what was tougher than you expected?

Martine: / What still stands out for me is the idea to create Idorsia. To develop a win-win situation for everyone; both companies, the employees, our shareholders and ultimately the patients, as it meant we could continue with our unique drug discovery approach and R&D pipeline. Of course, there were many challenges in the execution of the idea. For example, many of the compounds which stayed with Actelion were discovered or at least profiled in our labs. This meant that data going back 20 years needed to be transferred. This was a big challenge due to the sheer wealth of the data.

Guy: / I was astonished by how smooth the transition ran. This was only possible due to the meticulous planning by all involved during the few short months preceding "Idorsia Day 1". Nevertheless, there are always surprises and challenges, and I'm pleased to say that we were able to overcome all of them, including establishing new processes and a light structure, even with very restricted resources and limited manpower, and all that without putting any project at risk.

What do you consider to be Idorsia's key strengths in terms of R&D competencies and capabilities?

Guy: / We have a very promising pipeline of new compounds, that's a very good starting point! We also have a highly motivated team of people dedicated to delivering high quality in all they do. And I'm very happy that we have consciously kept our processes flexible so that we can advance our projects rapidly in a pragmatic way.

Martine: / For me, it's all about the people! We have a great team of brilliant scientists and while Idorsia is new, we have been a team for a long time. We have a common approach: novel projects that will answer medical problems in a truly groundbreaking way, balanced with less risky projects where we have a deep understanding of the disease mechanisms. Our people have the entrepreneurial spirit in their DNA and they are open to smart risk-taking.

Idorsia's pipeline is very diverse, covering multiple therapeutic areas. How did we end up with such diversity?

Martine: / Our strategy at Actelion was to diversify our product offering. We have intentionally built a pipeline that addresses different diseases that have no treatment, or where a group of patients is resistant to the treatment available. While we target a variety of diseases, the way we work in research is focused and built around our core competencies.

Guy: / We also tailor the target indication to characteristics of the compound. We always try to find the disease, spectrum of diseases or subset of medical conditions where the molecule will fit best from an efficacy and safety perspective, and where it addresses a medically important need.

"Diversity may bring logistical challenges, but it also offers a wider range of opportunities, ultimately this helps balance the inherent risk of a development pipeline and offers a greater chance of success.".

"We have a highly motivated team of people dedicated to delivering high quality in all they do... We are pragmatic, remain flexible, and focus on what is essential for success."

Where do you see the advantages of this diversity, and how do you manage the possible risks?

Martine: / If you pin all your hopes in one area, you become vulnerable to several external factors that are out of your control. You also miss out on the opportunity to bring major benefits to patients in other areas. We stay opportunistic to innovation.

We proactively manage our diversity though. Once we've entered into a new disease area or a new mechanism, we may explore further and leverage the newly-created expertise. That is how our current therapeutic areas have evolved.

Guy: / Diversity may bring logistical challenges, but it also offers a wider range of opportunities. Ultimately, this helps to balance the inherent risk of a development pipeline and offers a greater chance of success.

You intend to initiate four Phase 3 programs within the next 12 months. That is a mammoth task even for large established companies – how are you managing to do this as a start-up?

Guy: / We are pragmatic, remain flexible, and focus on what is essential for success. We have established a flat organizational structure to enable fast decision-making. We have highly committed people with core competencies, but acknowledge that we cannot do – and be excellent at – everything,

so we have adopted a flexible resource methodology, outsourcing operational tasks where appropriate. We are also able to adapt our way of working to the various characteristics of the advanced development projects, which are very different in size, length, and level of complexity.

"...it's all about the people! We have a great team of brilliant scientists and while Idorsia is new, we have been a team for a long time."

Looking at the early-stage compounds, where do you see the most promise?

Guy: / All projects are promising and risky in equal measure, they go hand in hand at this stage of development. Our new molecules have extraordinary properties. It is our task, collectively, to find the best development path for each of them. It is only at the end of the development that we will know which ones were the most promising.

Martine: / For me, all are exciting because they all bring a new approach to answering a medical problem and therefore could bring significant benefits to many patients.

Having an established group in drug discovery, there must be high expectations of delivery. Can you share anything about what we can expect in the future?

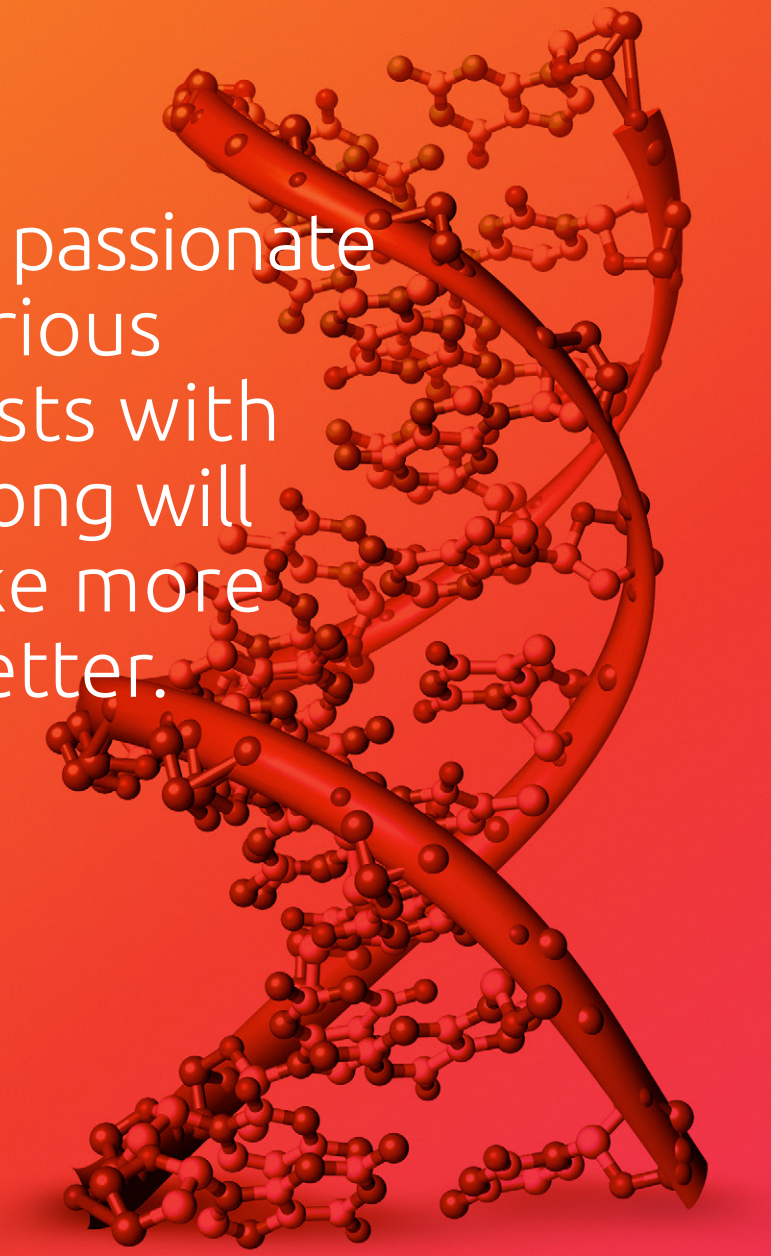
Martine: / Well, you can see that we have some front-runner compounds that are moving into late-stage clinical development. I've already mentioned that we keep our research within our fields of expertise to limit the risk of diversity. As a result, you can expect to see more compounds coming through that are active in the fields of CNS, immunology, cardiovascular and rare diseases. Of course, sometimes there are surprises, and our research takes us in a new direction. In that case, we may look for ways to maximize the impact of our compounds through partnerships, such as the one we announced in cancer immunotherapy with Roche at the end of 2017.

From a personal perspective, what was your most memorable Idorsia moment from experience so far?

Guy: / It's difficult to single out just one moment. Every day at Idorsia has been exciting, full of surprises and challenges. In fact the last six months will stay in my memory and I'm sure I'm not the only one who will say that. Above everything though, I find the happiness of teams when they see great study results most rewarding. Within weeks of Idorsia starting its operations, we saw the excellent results from our orexin receptor antagonist program. The perfect example of what can be achieved when you combine a great molecule, a scientifically sound program, and excellent study conduct.

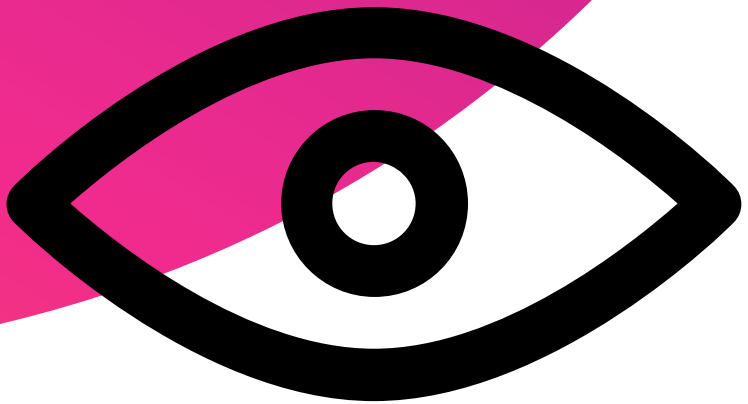
Martine: / I think back to the day of the announcement. To see the reaction of all Actelion employees as we were able to share our plans with them for the first time was extraordinary. After a period of uncertainty, there was considerable relief that there was a place for everyone. This was quickly followed by excitement. J&J has such a strong reputation as a caring company committed to patients, and there was a new venture to advance our pipeline. The excitement for the future was tangible.

We are passionate and curious scientists with the strong will to make more lives better.

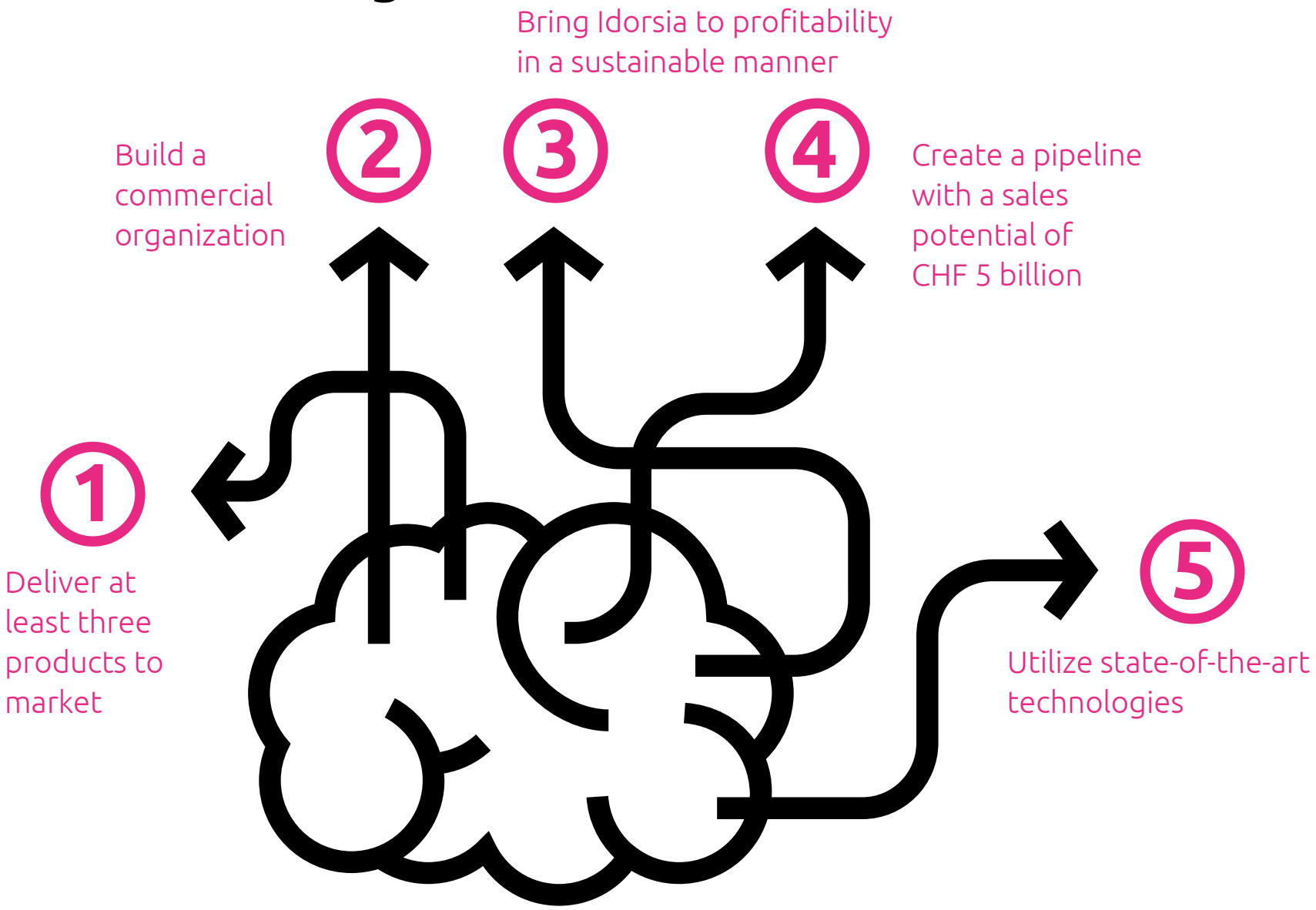


Our Strategic Priorities

5 **key priorities to ensure the company's success over the next 5 years.**



More power –
For scientific thinking



More innovation – Transforming the horizon

The purpose of Idorsia is to discover, develop and bring more, innovative medicines to patients. To achieve this, we will develop Idorsia into one of Europe's leading biopharmaceutical companies, with a strong scientific core.

We have identified five key strategic priorities to ensure the company's success over the next 5 years.

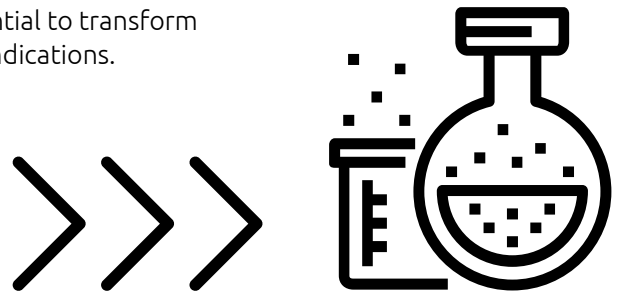
Promising compounds

1

Idorsia aims to deliver at least three products to market with the potential to significantly change treatment options in their target disease, resulting in assets with major commercial potential.

We have a diversified and balanced clinical development pipeline covering multiple therapeutic areas, including CNS, cardiovascular and immunological disorders, as well as orphan diseases.

We bring new perspectives to the development of innovative compounds, challenging accepted paradigms to answer the questions that matter to patients. Our key assets have the potential to transform treatment in the target indications.



From bench to bedside

2

Idorsia aims to build a commercial organization to maximize the value of our innovations.

A long-term strategic perspective is vital to ensure that our drugs address important healthcare challenges. A smart company must adopt creative approaches to delivering its medicines to patients.

To maximize the benefit of our pioneering therapies for patients, we will consider strategic partnerships and build our own commercial infrastructure to provide access to our drugs in certain therapeutic fields.

Becoming a sustainable company

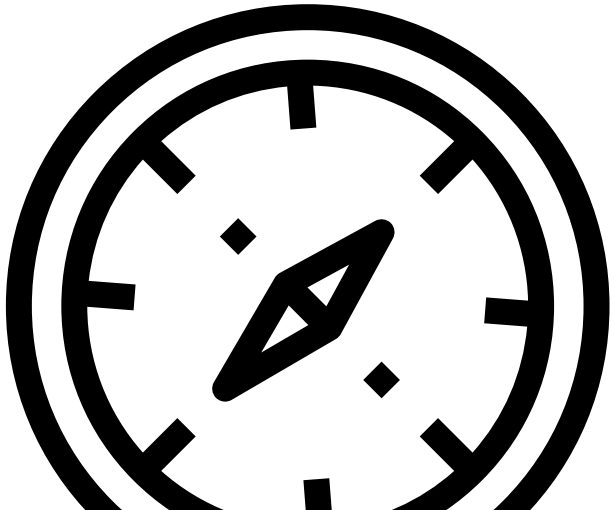
3

Idorsia's highly qualified professionals aim to rapidly advance the development pipeline and commercial readiness, so as to bring Idorsia to profitability within 5 years.

We are building Idorsia with a long-term focus and ambitious aspirations. We will run the company in a responsible and sustainable way. Becoming profitable will provide us with financial independence and secure our future.

Idorsia has the potential to generate significant revenues from product sales once the first of its pipeline candidates has received regulatory approval for commercialization. In the meantime, Idorsia has generated revenue through a milestone payment from its collaboration with Janssen on the co-development and commercialization of aprocitentan; this collaboration could also generate royalty payments. Furthermore, Idorsia could benefit from revenue-sharing payments relating to potential sales of ponesimod and cadazolid.

We are providing a supportive and stimulating environment for high-performing teams, recognizing and rewarding their contributions.



Building a bright future

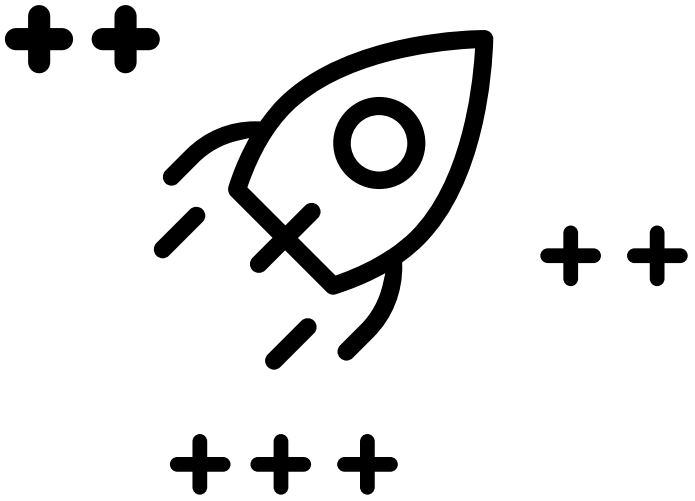
4

Idorsia aims to create a clinical development pipeline of assets with a sales potential of at least CHF 5 billion.

Idorsia is a high-potential biopharmaceutical company with a balanced portfolio of innovative drugs. As our assets advance and reach the market, there is a need to constantly replenish the pipeline with innovative compounds. This is where our highly productive drug discovery engine creates opportunities.

We are passionate and curious scientists, determined to help patients function better, feel better and live longer. Our drug discovery focuses on creating small molecules that are active on well-selected molecular targets. Knowledge built through the development of drugs active on one target can often benefit projects in several therapeutic areas.

Our focus on disorders with a high medical need means that our discoveries create significant value for patients, payors and society in general.



Mastering complexity without bureaucracy

Idorsia aims to increasingly utilize state-of-the-art technologies to aid discovery, development and commercialization of our innovative therapies.

As we want to remain at the cutting edge of science, it is vital that we consider innovative approaches from bench to bedside. We must integrate computational tools and digital technologies at different stages of the drug discovery, development and commercialization process, to maximize our potential and bring breakthrough medicines to patients.

Idorsia will look for creative ways to harness advances in technology to focus on novel targets and use new drug development methods. All functions of Pharmaceutical Development are streamlined to ensure the delivery of technically tailored and high-quality medicines. As the company establishes its commercial infrastructure, we will look for opportunities to use technology to benefit as many patients as possible.

When it comes to our colleagues' daily life at Idorsia, we also employ pragmatic solutions to minimize the need for bureaucracy. Continuous learning and work-life balance are important engagement drivers to ensure that everyone thrives at Idorsia.

We strive to be among the world's best pharmaceutical companies and the most stimulating places to work.

Our People

More experience – Driving innovation

Simply put – our success depends entirely on our people! This is why we want to recruit, engage, and develop talented people who are passionate about working together and applying science to bring benefits to patients.

A Manifesto for Success

It’s the Idorsia team’s passion for science that will translate our objectives into value creation for all our stakeholders.

We are doing more with less in a highly productive environment where people enjoy their work. Our Idorsia professionals show up with energy, intellect and creativity.

To reach our ambitious goals, we **advance** with energy and drive. We take full ownership and accountability to find solutions and outpace the competition.

Whatever the challenge, we are agile and **pragmatic** in implementing initiatives without compromising the quality of our work.

To seize more opportunities, we **invent** with creativity and imagination. Our work is science- and data-driven, and we remain open to new approaches in all aspects of what we do.

We **team up** to harness the power of our collective passion and sense of fun. We work collaboratively, sharing information and exchanging ideas, listening to and supporting each other.

We are curious, open-minded, and we **learn** continuously. We are encouraged to expand our knowledge, skills and self-awareness, while looking for ways to apply what we've learned.

Working at Idorsia

Our people are our most important asset, and fully leveraging their potential is critical to our success. We want them to feel proud of their work and the company they work for.

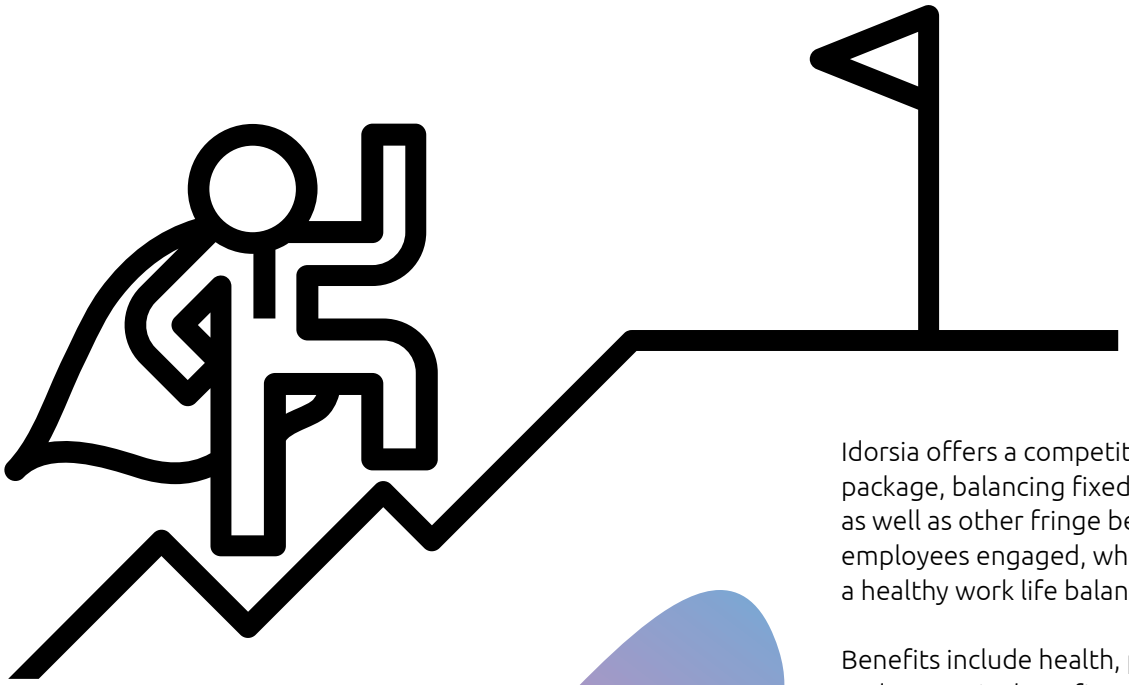
We take an integrated approach to rewards and talent management, designed to build an organization of highly engaged and enthusiastic professionals. As a result, our people are committed to making Idorsia one of Europe's leading biopharmaceutical companies, while at the same time growing both personally and professionally.

Diversity & Inclusion
We are committed to fostering respect, fairness and equal opportunities for all our employees, as we believe it is vital to create and support a diverse and inclusive workplace. Idorsia does not tolerate discrimination of any kind.

We are committed to harnessing the power of difference to achieve business success, and we are implementing strategies to attract, retain and advance top talent from all backgrounds and cultures. During the recruitment process, we seek to attract a diverse pool of candidates, focusing on the skill-set they offer and matching their competencies to the behaviors we expect our people to live by daily.

Recruitment, Development & Retention
We seek to recruit, develop and retain talented individuals who want to make a difference to the lives of patients across the globe. We regularly assess our talent to identify high performance and provide support for those who display potential for further growth – e.g. through further education, leadership training and on-the-job assignments.

To support people in achieving their full potential, we provide a range of internal and external learning and development programs. All our employees own their development in partnership with their line manager and Human Resources. For example, we emphasize results-oriented coaching, encourage internal mentorship, offer a variety of training programs, and fully support language-learning.



Idorsia offers a competitive total rewards package, balancing fixed and variable pay, as well as other fringe benefits, to help keep employees engaged, while also maintaining a healthy work life balance.

Benefits include health, pension, disability and maternity benefits. Depending on local requirements, additional benefits may be available.

At our headquarters, we offer subsidized on-site daycare for children from age 3 months to 6 years.

Recognition

Employee recognition, when delivered effectively, is a powerful engagement tool for driving desired behaviors and achieving organizational goals. Our cutting-edge people management practices transparently align our work with the company's strategy, fostering a culture of continuous feedback.

We recognize extraordinary achievements and emphasize the importance of working in teams, while providing meaningful development opportunities.

Our simple and transparent reward and recognition philosophy is based on engaging everyone in an entrepreneurial approach to long-term value creation.

The Idorsia Leadership Team

Idorsia has a strong and visionary leadership team, with the power and drive to create more remarkable innovations and more new medicines.

From day one of Idorsia, it was clear that the management team were trying to create something new and different. Idorsia has great potential to be a special place where groundbreaking discoveries are made. The management understood that this potential will only be realized if we create an environment that encourages creative thinking, and if people are empowered to challenge existing paradigms.

In this start-up phase of Idorsia, we all depend on the results that we achieve collectively. Management has therefore put systems in place to recognize contribution, encourage high performance and share the results that we achieve individually and collectively.

They have also acknowledged that it's not just what we achieve, but how we get there. To support this, management have identified model behaviors which will help us to implement our strategy, shaping Idorsia's corporate culture.

There is a tangible determination to work collaboratively to progress our drug portfolio and build a commercial infrastructure.

Members of the Idorsia Executive Committee

"Idorsia is making good progress in advancing its pipeline and in separating from Actelion."

André C. Muller
Executive Vice President, Chief Financial Officer

"Each day we aim for more. We keep improving and continue learning."

Martine Clozel
Executive Vice President, Chief Scientific Officer

"We are passionate about pragmatic science."

Guy Braunstein
Executive Vice President, Head of Global Clinical Development

"We are working hard to advance our highly innovative pipeline and further enhance its value proposition."

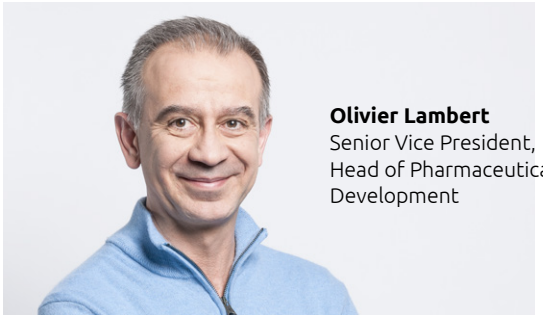
Jean-Paul Clozel
Chief Executive Officer

More agile – Mastering complexity

Andrew C. Weiss
Senior Vice President,
Head of Investor Relations & Corporate Communications

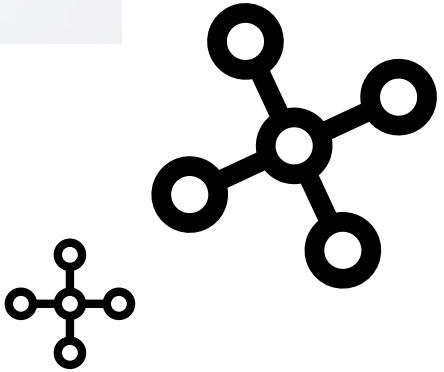
Alexander Khatuntsev
Senior Vice President,
Head of Human Resources

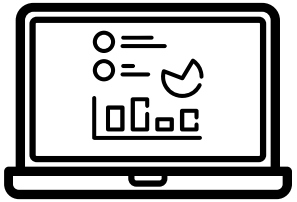
Oliver Peinelt
Senior Vice President,
Group General Counsel



Olivier Lambert
Senior Vice President,
Head of Pharmaceutical Development

Thomas Weller
Senior Vice President,
Head of Drug Discovery Chemistry





Markus A. Riederer
Senior Vice President,
Head of Drug Discovery Biology

Ulrich Mentzel
Senior Vice President,
Head of Drug Discovery Pharmacology & Preclinical Development

Engagement is a core element of the Idorsia culture: We are all committed to going the extra mile – Reaching out for more.

Our Responsibilities

7 **main topics are covered by Idorsia's corporate responsibility efforts.**

More ideas – Changing the world

Idorsia's corporate responsibility efforts encompass employee development and diversity, governance and ethics, as well as the organization's economic, environmental, and social performance.

Privacy & Data Security

Corruption & Bribery

Labor & Human Rights

Research Ethics

Clinical Trials Transparency

Stakeholder Engagement

Environment

More drive – For a better future

Idorsia's stakeholders value excellence in governance. We aim to be transparent, applying the highest standards of integrity across the company.

Our Legal & Compliance function seeks to drive a culture of integrity, with the following priorities:

- communicate clearly to employees
- focus on key compliance risk areas
- improve compliance behavior through training and support
- ensure that employees can raise concerns and that these are properly addressed
- ensure fair and objective investigations of potential policy breaches

An example of best practice in governance is our Code of Business Conduct, which underpins our commitment to integrity and sets out fundamental rules for interacting with others as we drive our business forward. Supporting policies, standard operating procedures and guidelines provide more detail on how our high-level commitments are to be applied in practice.

All Idorsia employees have undergone mandatory training to ensure compliance with the Code.

The Idorsia Code of Business Conduct is at the foundation of our corporate culture and defines the core principles and ethical standards by which we create value in our company. It is the responsibility of every Idorsia employee to be familiar with, and to comply with, our Code.

We are proactive in establishing policies and practices that support strong corporate governance and transparency. These policies and practices are continually reviewed and enhanced as appropriate.

Privacy & Data Security

Idorsia understands the importance of protecting personal information and applying the highest ethical and regulatory standards. We are committed to respecting our stakeholders' privacy and safeguarding their personal information. Idorsia's data protection policy covers all personal data on study participants, healthcare professionals, customers, suppliers and employees.

To ensure the integrity and privacy of personal and health-related information provided to us, we use state-of-the-art information security programs, focusing on protection of sensitive information and detection of unauthorized access.

Corruption & Bribery

Full compliance with applicable laws in all the regions where Idorsia operates is crucial to our success. Idorsia's position is clearly stated in our Anti-Corruption and Anti-Bribery Policy: We take a zero-tolerance approach to bribery and corruption and are committed to acting professionally in all our business dealings and relationships wherever we operate.

We implement and enforce effective systems to counter bribery. We will uphold all laws relevant to countering bribery and corruption in all the jurisdictions in which we operate. All Idorsia employees are required to undergo training on this policy.

Labor & Human Rights

Idorsia respects the rights of its employees as set out in the Universal Declaration of Human Rights, and we fully comply with all relevant laws, rules and regulations governing labor, employment and the employment relationship in all the countries where we operate.

We respect the right of all employees to join a legally recognized employee association, and we comply with all laws relating to employee representation. We strive to maintain an open dialogue with all our employees and their representatives.

Due to the nature of our business and the location of our operations, the risk of child or forced labor is minimal. We do, however, remain vigilant for unexpected issues that may arise – not only in our own operations but also in relation to our procurement practices. Our expectations regarding working conditions, human rights protection, business ethics, legal compliance and environmental protection are set out in our Code of Conduct.

Research ethics

We strive to maintain the highest ethical, scientific and clinical standards in all our research activities, and to comply with all national and international standards. Idorsia regularly reviews its research policies to align them with its strategic objectives, and with the evolving values and goals of stakeholders.

Regulatory authorities around the world require pharmaceutical companies to test all new drugs before they are launched, and there is no alternative to including some animal testing as part of this process. This is essential both for scientific reasons and to safeguard the volunteers and patients who take part in subsequent clinical trials. As a fundamental principle, we support the "three R's" in relation to animal testing:

Refinement – Alleviate or minimize impacts to animals by reducing potentially painful or invasive procedures, whenever possible.
Reduction – Use the absolute minimum number of animals required to obtain valid results in each study.
Replacement – Always look for alternative, non-animal-based research methods where possible.

The number of animals used in drug development has dropped dramatically over the past three decades as a result of industry initiatives like this. Idorsia has a strong policy on the care, welfare and treatment of animals, and we conduct regular audits to make sure that our expectations are being met, whether the studies are being conducted in-house or outsourced.

In addition, we ensure that the use and care of all laboratory animals meets or exceeds relevant local, national and international regulations. Our programs and facilities are subject to unannounced regulatory review and inspections. For sponsored work at contract research organizations, our animal welfare oversight activities include regular on-site evaluations by our veterinary staff.

Idorsia will never use great apes (gorillas, chimpanzees, orangutans and bonobos) in its research.

Clinical Trials Transparency

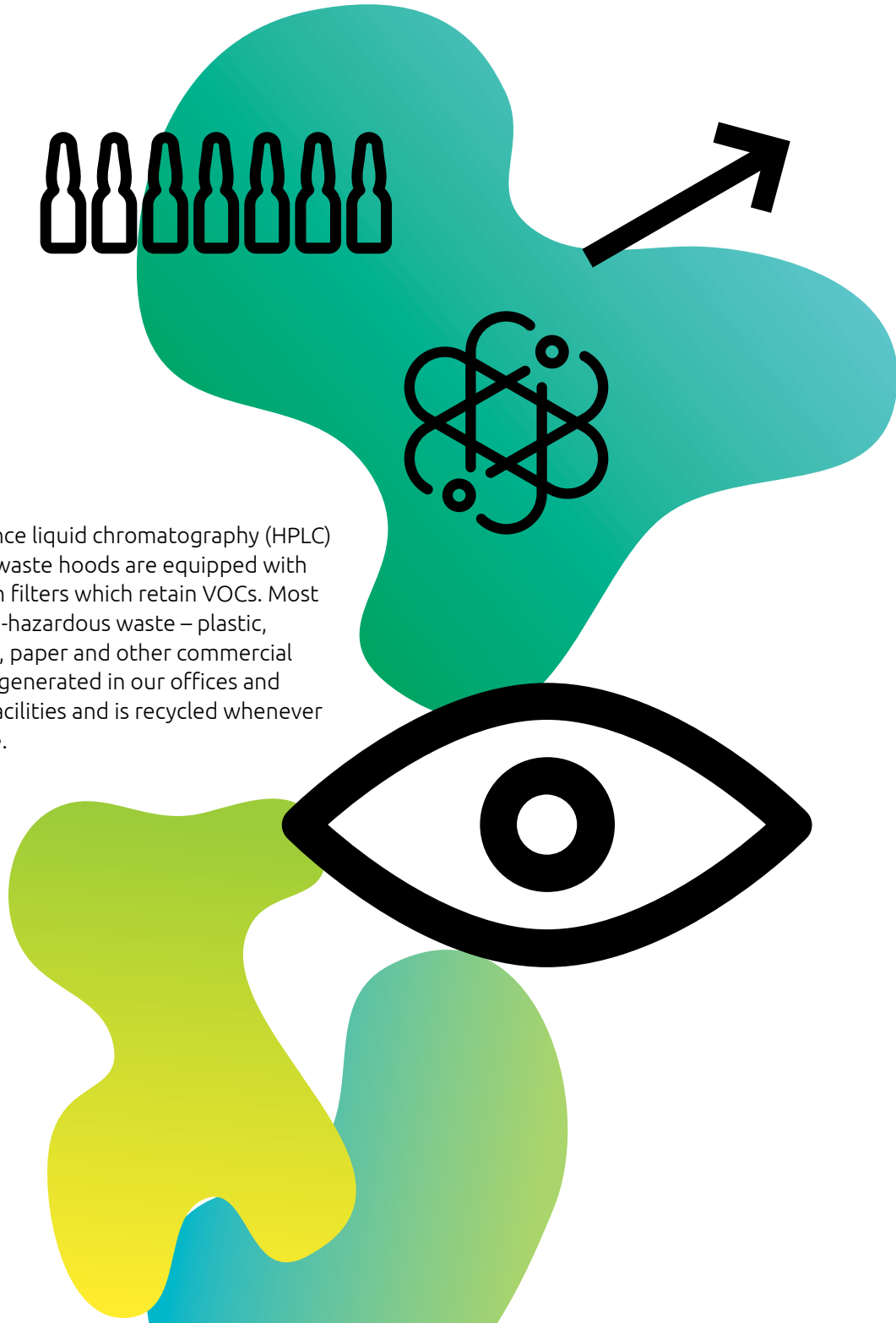
Clinical trials are essential to the development of innovative medicines. We are committed to the highest standards of quality and ethical conduct in all our clinical research. Our trials are performed in accordance with internationally accepted guidelines, and protocols are evaluated by independent review boards and ethics committees prior to study initiation. We are also dedicated to improving public health through responsible clinical trial data transparency which – while complying with applicable regulations – respects our proprietary information and patients' privacy.

Stakeholder Engagement

We seek transparency and dialogue with all stakeholders to improve our understanding of their needs. These stakeholders include patient associations, healthcare providers, shareholders, employees, authorities, the environment and local communities. We consult and engage with all our stakeholders on a regular basis and incorporate their feedback into the company's strategy and risk management.

Environment

Idorsia's primary environmental sustainability goal is to reduce our environmental impact to help ensure a safe and healthy environment for present and future generations. Due to the nature of our activities, our environmental footprint is not as substantial as in other industries. However, we take our responsibility very seriously and are continuously working to further reduce the environmental impact of our operations.



Although our business is not water-intensive, we are continuously working to minimize the use of this precious resource. Water is used for a variety of purposes, such as laboratory experimentation, drinking water, facility cooling, and cleaning and maintenance operations. Our facilities are built to the latest standards and include many features designed to minimize water use (e.g. tap sensors). We strictly adhere to all regulations concerning water quality and potential impacts on water resources.

We work to eliminate waste as far as possible. When this is not possible, we aim to reduce waste and recycle to mitigate environmental impacts. Idorsia's hazardous waste (as defined by local regulations) stems from our research activities in Switzerland. It is sent off-site for recycling, reuse, treatment or disposal. Volatile organic compounds (VOCs), used in our research facilities, may have a negative impact on the environment. To minimize emissions of VOCs, our high-performance liquid chromatography (HPLC) units and waste hoods are equipped with absorption filters which retain VOCs. Most of our non-hazardous waste – plastic, cardboard, paper and other commercial waste – is generated in our offices and catering facilities and is recycled whenever possible.le.

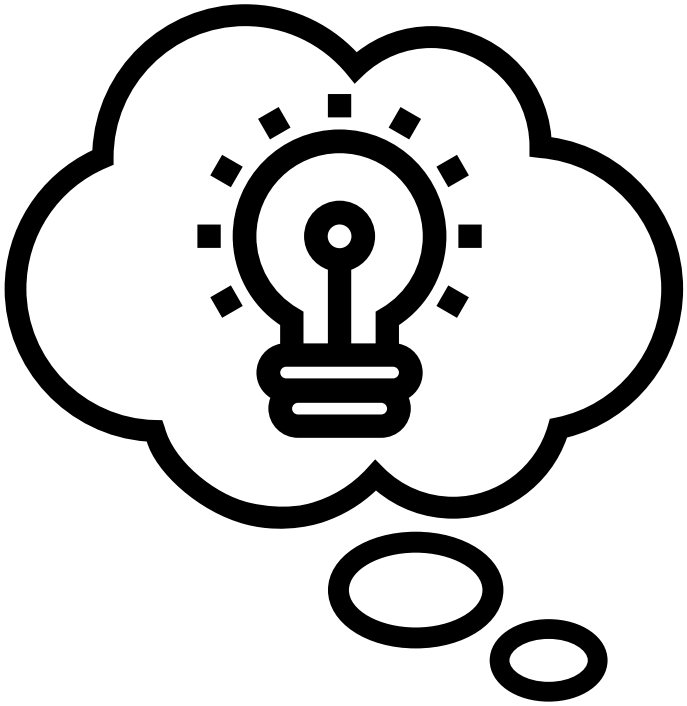
More hope - Inspiring young minds

We aim to be good neighbors in the communities where we live and work. Our community efforts focus primarily on science education, with the goal being to ignite a passion for science in young minds.

We have partnered with our neighbor Actelion to invest in children's education. This year, we invited over 150 youngsters and teachers from local schools to our "Elementary school science days". At this 2-day event, young students are exposed to hands-on, real-world science, with a fun and interactive approach.

This year also saw over 170 local high school students attending our series of summer lectures, featuring topics such as "High-tech in research", "The fate of a substance in the body", "Providing insights into genetics and applied genomics", "Cancer – pathogenesis and treatment options", "Clinical studies in healthy volunteers", and "The long way to the market: clinical trials in patients".

The feedback was incredibly positive, not only from the students and teachers attending, but also from our volunteers, who enjoyed taking time out of their busy days to help inspire the next generation.



"It's very rewarding to see the youngsters having so much fun in the lab. If we inspire just a few of them to pursue a career in science, then it's worth all the effort."

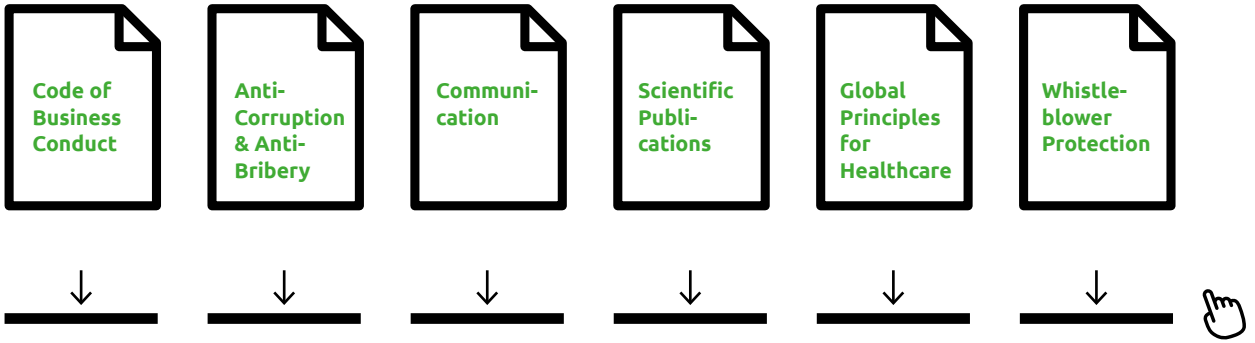
Matthias Merrettig
Lab Expert, Chemistry

More authentic – Culture of transparency

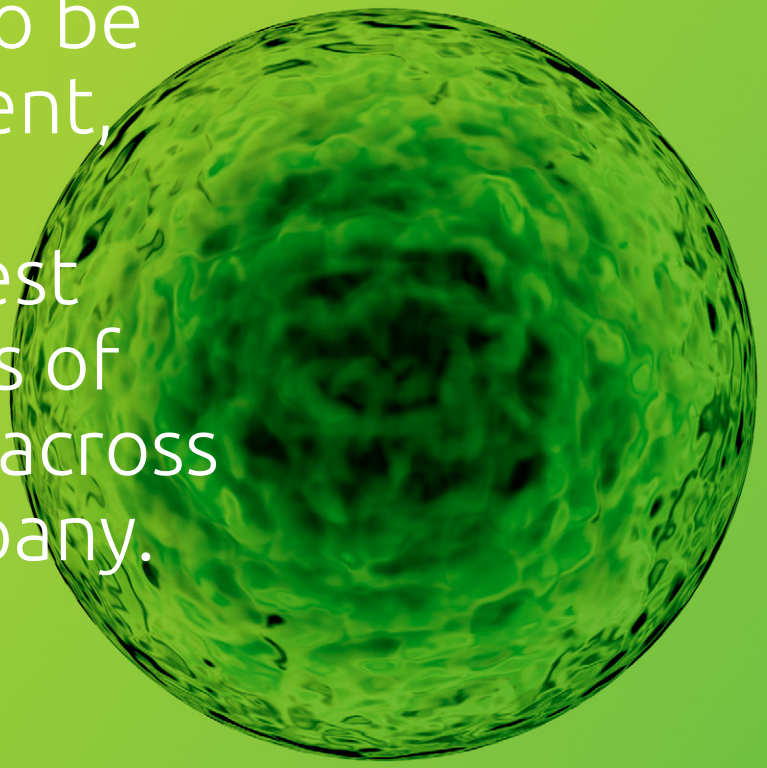
Idorsia is proud to be working in a highly regulated industry with codes of conduct, guidelines and regulations that are designed to protect patients, healthcare professionals and the reputation of the industry.

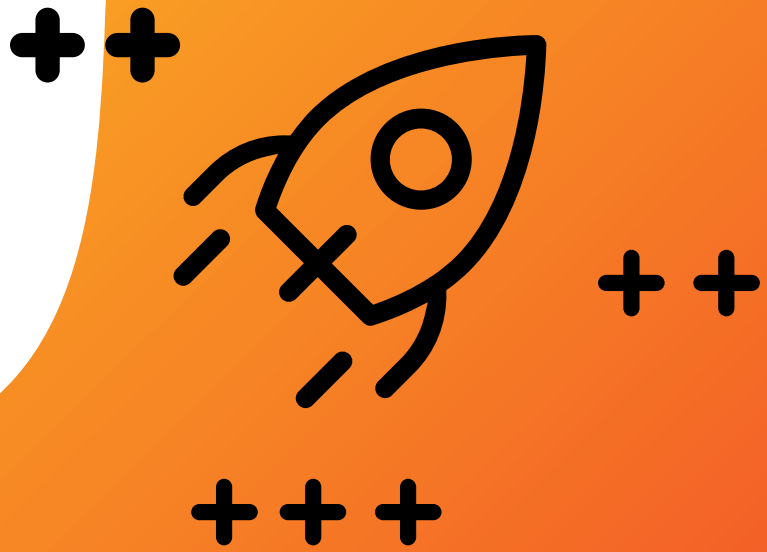
To ensure that our employees understand how important it is to work in a compliant, ethical and transparent manner, Idorsia has implemented a number of policies and guidelines covering the breadth of its business areas. Access to these documents is ensured for all employees via an electronic quality management system.

Policies which are relevant to other stakeholders are available on our corporate website and can be downloaded below.



We aim to be transparent, applying the highest standards of integrity across the company.





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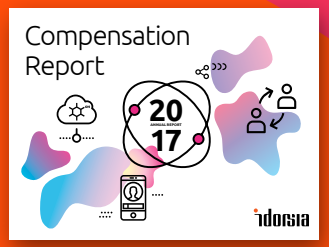

Curious to learn more?
Reach out to us.

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