# Media Release
# February 6, 2020

## Idorsia announces financial results for 2019 – clinical programs advanced markedly – approaching first key results

**Allschwil, Switzerland – February 6, 2020**
Idorsia Ltd (SIX: IDIA) today announced its financial results for the full year 2019.

### Operational updates

- Delivering innovation – two new compounds entering clinical development
- Advancing all our clinical programs – first Phase 3 results expected soon
- Building up our infrastructure – planning for the launch of our first product
- Patricia (Patty) Torr appointed as President of our US commercial organization

### Financial updates

- US GAAP operating expenses 2019 at CHF 506 million
- Non-GAAP operating expenses 2019 at CHF 470 million
- Guidance for 2020: US GAAP operating expenses of around CHF 540 million and non-GAAP operating expenses of around CHF 500 million (both measures exclude any potential milestone payments).

**Jean-Paul Clozel, MD and Chief Executive Officer, commented:**
"2019 was another exciting year for Idorsia, we began building our commercial capabilities and started to plan the launch of our first product. We also advanced each of our clinical programs, with the first Phase 3 results expected soon. Furthermore, we delivered more innovation through our drug discovery organization by adding two new compounds to our pipeline. Since the results of the daridorexant program are just months away, 2020 promises to be every bit as exciting."

**Simon Jose, Chief Commercial Officer, added:**
"Over the last 12 months we have been focusing on establishing our core commercial team and defining strategies for our key late-stage compounds. We have hired several people to fill critical roles, most recently appointing Patty as President of our US commercial organization, enabling us to step up our pre-launch activities for daridorexant in the US as soon as Phase 3 results are available. We have been very fortunate to attract experienced individuals with diverse backgrounds, all with the creativity which is a hallmark of Idorsia."

### Financial results

| **US GAAP results** | **Full Year** | | **Fourth Quarter** | |
|---|---|---|---|---|
| **in CHF millions, except EPS (CHF) and number of shares (millions)** | **2019** | **2018** | **2019** | **2018** |
| Revenues | 24 | 61 | 4 | 41 |
| Operating expenses | (506) | (432) | (131) | (141) |
| Operating income (loss) | (482) | (371) | (127) | (101) |
| Net income (loss) | (494) | (386) | (142) | (108) |
| Basic EPS | (3.76) | (3.10) | (1.08) | (0.83) |
| Basic weighted average number of shares | 131.2 | 124.8 | 131.2 | 131.1 |
| Diluted EPS | (3.76) | (3.10) | (1.08) | (0.83) |
| Diluted weighted average number of shares | 131.2 | 124.8 | 131.2 | 131.1 |

US GAAP revenue of CHF 24 million in 2019 related to deferred contract revenue recognized in connection to the collaboration agreements with Janssen (CHF 19 million) and Roche (CHF 5 million), compared to a revenue of CHF 61 million in 2018.

US GAAP operating expenses in 2019 amounted to CHF 506 million (of which CHF 439 million R&D and CHF 68 million SG&A expenses), whilst operating expenses in 2018 amounted to CHF 432 million (of which CHF 370 million R&D and CHF 61 million SG&A expenses).

US GAAP net loss in 2019 amounted to CHF 494 million compared to CHF 386 million in 2018. The increase of the net loss was mainly driven by higher operating costs.

The US GAAP net loss resulted in a net loss per share of CHF 3.76 (basic and diluted) in 2019 compared to a net loss per share of CHF 3.10 (basic and diluted) in 2018.

| **Non-GAAP* measures** | **Full Year** | | **Fourth Quarter** | |
|---|---|---|---|---|
| **in CHF millions, except EPS (CHF) and number of shares (millions)** | **2019** | **2018** | **2019** | **2018** |
| Revenues | 24 | 61 | 4 | 41 |
| Operating expenses | (470) | (399) | (122) | (133) |
| Operating income (loss) | (446) | (339) | (118) | (92) |
| Net income (loss) | (448) | (340) | (121) | (91) |
| Basic EPS | (3.41) | (2.72) | (0.92) | (0.70) |
| Basic weighted average number of shares | 131.2 | 124.8 | 131.2 | 131.1 |
| Diluted EPS | (3.41) | (2.72) | (0.92) | (0.70) |
| Diluted weighted average number of shares | 131.2 | 124.8 | 131.2 | 131.1 |

** Idorsia measures, reports and issues guidance on non-GAAP operating performance. Idorsia believes that these non-GAAP financial measurements more accurately reflect the underlying business performance and therefore provide useful supplementary information to investors. These non-GAAP measures are reported in addition to, not as a substitute for, US GAAP financial performance.*

Non-GAAP net loss in 2019 amounted to CHF 448 million: the CHF 46 million difference versus US GAAP net loss was mainly due to depreciation and amortization (CHF 20 million), share-based compensation (CHF 17 million), a negative non-cash financial result (CHF 2 million), and a negative non-cash tax result (CHF 8 million).

The non-GAAP net loss resulted in a net loss per share of CHF 3.41 (basic and diluted) in 2019 compared to a net loss per share of CHF 2.72 (basic and diluted) in 2018.

**André C. Muller, Chief Financial Officer, commented:**
"With a cost-conscious attitude and a slight shift in timelines for some clinical programs, we spent less in 2019 than originally expected, ending the year with liquidity of CHF 739 million. For 2020, we expect non-GAAP operating expenses to be around CHF 500 million, excluding unforeseen events and potential milestone payments. Idorsia's liquidity will not last until break-even, thus we will need additional funding to bring our products to market, but we are fortunate in having several unencumbered assets in clinical development with key results in the near future, as well as financing options available to us."

## Liquidity and indebtedness

At the end of 2019, Idorsia's liquidity (including cash, cash equivalents, short- and long-term deposits) amounted to CHF 739 million.

| (in CHF millions) | Dec 31, 2019 | Sep 30, 2019 | Dec 31, 2018 |
|---|---|---|---|
| **Liquidity** | | | |
| Cash and cash equivalents | 263 | 385 | 799 |
| Short-term deposits | 476 | 490 | 123 |
| Long-term deposits | - | - | 298 |
| **Total liquidity*** | **739** | **875** | **1,220** |
| | | | |
| **Indebtedness** | | | |
| Convertible loan | 380 | 378 | 372 |
| Convertible bond | 199 | 199 | 198 |
| Other financial debt | - | - | - |
| **Total indebtedness** | **579** | **577** | **571** |

*rounding differences may occur

## Clinical Development Pipeline

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

The Phase 3 registration program investigating daridorexant (10, 25, and 50mg) for the treatment of adult and elderly patients with insomnia, has completed recruitment of the two pivotal studies. Results of the first study, investigating daridorexant doses 25 and 50mg, are expected in the second quarter of 2020.

Progress was also made with our early-stage pipeline by bringing two new compounds into Phase 1 development in the field of Immunology and Immunology / Cancer immunotherapy.

Further details of the pipeline can be found in our clinical development fact sheet.

| Compound | Mechanism of Action | Target Indication | Status |
|---|---|---|---|
| **Daridorexant** | Dual orexin receptor antagonist | Insomnia | Phase 3 – recruitment complete |
| **Aprocitentan*** | Dual endothelin receptor antagonist | Resistant hypertension management | Phase 3 |
| **Clazosentan** | Endothelin receptor antagonist | Vasospasm associated with aneurysmal subarachnoid hemorrhage | Phase 3 |
| **Lucerastat** | Glucosylceramide synthase inhibitor | Fabry disease | Phase 3 |
| **Cenerimod** | $S1P_1$ receptor modulator | Systemic lupus erythematosus | Phase 2 |
| **Selatogrel** | $P2Y_{12}$ receptor antagonist | Suspected acute myocardial infarction | Phase 2 – complete |
| **ACT-774312** | CRTH2 receptor antagonist | Nasal polyposis | Phase 2 |
| **Sinbaglustat (ACT-519276)** | GBA2/GCS inhibitor | Rare CNS diseases | Phase 1 |
| **ACT-539313** | Selective orexin 1 receptor antagonist | Psychiatric disorders | Phase 1 |
| **ACT-709478**** | T-type calcium channel blocker | Epilepsy | Phase 1 |
| **ACT-1004-1239** | - | Immunology / Cancer immunotherapy | Phase 1 |
| **ACT-1014-6470** | - | Immunology | Phase 1 |

** In collaboration with Janssen Biotech Inc. to jointly develop and solely commercialize aprocitentan worldwide*
*** Idorsia has granted to Neurocrine Biosciences, Inc. an option to license ACT-709478, this option will expire 30 days after the IND application acceptance by the FDA, expected in mid-2020*

*Idorsia has the option to license vamorolone from ReveraGen Inc. and has granted to Santhera Holding Ltd. the option to sub-license vamorolone worldwide (except Japan and South-Korea) for all indications.*

## Human Resources

Idorsia created 63 new positions worldwide in 2019, bringing the total number of employees (permanent, post-doc, and apprentices) to 816 (2018: 753).

## Annual Report

Full details on the progress made in 2019 are available in Idorsia's 2019 Annual Report, at www.idorsia.com/annual-report

## Note to Shareholders

The Annual General Meeting (AGM) of Shareholders to approve the Annual Report of the year ending December 31, 2019 will be held on Wednesday May 13, 2020.

Registered shareholders with voting rights individually or jointly representing at least 5% of the share capital of the company, being entitled to add items to the agenda of the general meeting of shareholders, are invited to send in proposals, if any, to Idorsia Ltd, attention Corporate Secretary, Hegenheimermattweg 91, CH-4123 Allschwil, to arrive no later than March 24, 2020. Any proposal received after the deadline will be disregarded.

In order to attend and vote at the Annual General Meeting, shareholders must be registered in the company's shareholder register by May 4, 2020 at the latest.

## Results Day Center

Investor community: To make your job easier, we provide all relevant documentation via the Results Day Center on our corporate website: www.idorsia.com/results-day-center.

## Upcoming Financial Updates

- First Quarter 2020 Financial Results reporting on April 23, 2020
- Annual General Meeting of Shareholders on May 13, 2020
- Half-Year 2020 Financial Results reporting on July 23, 2020
- Nine-months 2020 Financial Results reporting on October 22, 2020

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## Notes to the editor

### Letter to Shareholders (as published in Idorsia's 2019 Annual Report on February 6, 2020)

**More energy – Growing and delivering**

Dear Shareholders,

Nearly three years ago, we launched our new enterprise with the ambition of making Idorsia one of Europe's leading biopharmaceutical companies. This year – as our detailed **Review of 2019** shows – we have made significant progress toward this goal by:

- ***delivering*** innovation through our drug discovery organization, with two new compounds entering clinical development;
- ***advancing*** all our clinical programs, with our first Phase 3 results expected soon; and
- ***building up*** our infrastructure and starting to plan for the commercial launch of our first product.

None of these achievements would have been possible without the full commitment of everyone at Idorsia. However, we are well aware that we cannot develop everything on our own, so, in 2020, we will focus on building the right partnerships and refining our strategic priorities, so that we can achieve our goals of financial sustainability and long-term value creation by delivering best-in-class, innovative drugs to patients.

We thank you for your confidence.

Sincerely,

Jean-Paul Clozel, Chief Executive Officer  Jean-Pierre Garnier, Chairman of the Board

## Chairman and CEO's Review of 2019 (as published in Idorsia's 2019 Annual Report on February 6, 2020)

In 2019, our focus has been on delivering on our strategic priorities by pressing ahead with our key clinical activities and getting ready for the wave of results approaching soon.

Having initiated four Phase 3 programs in 2018 – an impressive accomplishment for a company operating with a lean development team – we have been striving in 2019 to advance these programs as rapidly as possible, without compromising on quality. Each Phase 3 program, of course, involves company-wide activities to prepare for regulatory approval and launch.

We have set ourselves ambitious goals, seeking to achieve something which is exceedingly difficult to accomplish in our industry. So how have we done so far?

**Advancing the late-stage pipeline**

Thanks to our teams' hard work, the Phase 3 studies are running smoothly, with initial data anticipated in a matter of months. The first data reported will be for daridorexant, our dual orexin receptor antagonist for the treatment of insomnia. What potentially differentiates daridorexant from existing treatments is the delivery of clinically meaningful benefits in sleep onset and maintenance, with a duration of action designed not to exceed a normal night. Patients with insomnia often face multiple challenges, including both falling asleep and staying asleep. They are actively seeking new safe and effective treatment options which can address both these needs, thus helping them to function better during the day. By blocking the action of orexin, it is hoped that daridorexant will allow patients to sleep throughout the night, while avoiding the rebound, withdrawal, or tolerance problems associated with many sleep medications that act through broad sedation of the brain.

Later in 2020, we also expect to have the results of the Japanese registration study on clazosentan – a selective endothelin (ETA) receptor antagonist being developed for the reduction of vasospasm, and vasospasm-related morbidity and mortality, following aneurysmal subarachnoid hemorrhage (aSAH). This is a significant problem in Japan, where the prevalence of aSAH is around twice as high as in the rest of the world. Here too, we are eager to see the data, which could lead to a new treatment making a big difference for patients. The global Phase 3 study outside of Japan – for prevention of clinical deterioration due to vasospasm-related delayed cerebral ischemia following aSAH – is progressing well, with results expected around a year after the Japanese data.

Also moving forward, though not quite as quickly as initially hoped, is the development of lucerastat, an oral therapy offering a new treatment approach for all patients with Fabry disease, irrespective of mutation type. Lucerastat acts by reducing the damaging build-up of lipids which is responsible for all the symptoms of Fabry disease. The main objective in our registration study is the reduction of neuropathic pain – a symptom having a severe impact on many patients' lives. Additional endpoints include the reduction in gastrointestinal symptoms and the effect on biomarkers of Fabry disease. The results are now expected in 2021.

Our fourth Phase 3 program involves aprocitentan – an orally active dual endothelin receptor antagonist – investigated in the PRECISION trial for patients whose blood pressure is uncontrolled despite the use of at least three antihypertensive drugs. To accelerate recruitment, we are currently increasing the number of sites participating in this registration study. In addition, we recently decided not to proceed with a study of aprocitentan planned for patients with chronic kidney disease having uncontrolled blood pressure despite the use of antihypertensive drugs. Sites and patients that were to participate in this study can now be included in PRECISION. These measures should help speed up the evaluation of this new option, tapping the therapeutic potential of blocking the endothelin pathway.

**Engaging with the expert community**

A vital aspect of our work is ensuring that experts are aware of our research and understand how treatments are being advanced in their field. We achieve this through publications in peer reviewed journals and contributions to scientific conferences. Given the fierce competition for the limelight, only the most impressive results are selected for oral, rather than poster, presentations. In 2019, we were honored to be able to share the results of several of our programs with experts at the most prestigious medical conferences.

Phase 2 data for daridorexant was presented at the annual SLEEP meeting of the Associated Professional Sleep Societies, attended by around 5,000 delegates, and again at World Sleep 2019, the biennial congress of the World Sleep Society, with over 3,300 attendees. Phase 2 data for both aprocitentan and selatogrel was presented at the European Society of Cardiology (ESC) Congress 2019, attended by almost 35,000 experts. In addition, Phase 2 data for cenerimod was presented at the annual meeting of the American College of Rheumatology, with over 15,000 delegates.

It was also gratifying to see our partner Johnson & Johnson reporting data from a successful Phase 3 trial on ponesimod (for the treatment of relapsing multiple sclerosis) at the 2019 Congress of the European Committee for Treatment and Research in Multiple Sclerosis – the largest annual international meeting devoted to basic and clinical research in this field. Not only are the scientists who discovered ponesimod now with Idorsia, but our revenue-sharing agreement with J&J means that quarterly payments of 8% of the net sales of ponesimod products are likely to be our first source of regular income.

**Progress with mid-stage assets**

Cenerimod, our selective S1P1 receptor modulator, is being investigated for the treatment of adults with systemic lupus erythematosus in a multiple-dose efficacy and safety study; good progress is being made with recruitment, which was initiated at the beginning of 2019. Within the limited current treatment landscape, the properties of cenerimod and the mechanism of S1P1 receptor modulation provide significant potential to address the pathophysiology of lupus. We are doing everything we

can to move the study forward, and preparing future development in parallel, so that this important treatment can be brought to patients as quickly as possible.

In 2019, we also made significant progress with selatogrel, our highly selective P2Y12 receptor antagonist, which is being evaluated for an innovative approach to the management of acute myocardial infarction. In patients suffering a heart attack, subcutaneously administered selatogrel shows a very rapid onset of action (within 15 minutes), with the effects lasting over 4–8 hours. Given this rapid onset and the duration of action, as well as the safety and tolerability profile, we believe that selatogrel could be self-administered at the onset of symptoms to stop a suspected heart attack and preserve muscle and heart function. As we prepare the clinical evidence to support the use of selatogrel in these patients, we have been looking for a safe and reliable device which is easy to use under stressful conditions. In late 2019, we signed a deal with Antares Pharma, Inc., to develop a novel drug-device product combining selatogrel with the subcutaneous QuickShot® auto-injector. Usability and reliability studies are now being planned, and the Phase 3 design is being discussed with health authorities; the registration study is expected to be initiated in the first half of 2021. The potential importance of this product – both for patients and for the future of our company – cannot be overestimated.

**Preparing for success**
As the pipeline advances, we hope it will soon be time for Clinical Development to pass the baton to our commercial team. This year, our Chief Commercial Officer, Simon Jose, has been establishing his core commercial team and defining detailed commercial strategies for our key late-stage compounds.

The breadth of our late-stage pipeline is an amazing opportunity, but it also presents challenges for a small team. For example, our products span a variety of therapeutic areas, each requiring a different commercial strategy. A rare disease managed by specialists calls for a different approach than a more common disorder mostly managed by general practitioners, or a product intended for use in an intensive care hospital setting. To build these different strategies, we need a deep understanding of patients, prescribers and market landscapes, to see how we can best differentiate our products and communicate a clear, consistent message. Having performed our research and honed our plans, we are ready to rapidly integrate the study data and execute our strategies as soon as the results are available.

While our late-stage compounds are being developed into commercial products, we are also continuing to innovate – balancing novel projects, seeking to address medical needs in a groundbreaking way, with best-in-class projects, driven by our deep understanding of disease mechanisms.

**About Patricia Torr**
Patricia (Patty) Torr brings over 20 years of broad leadership experience and industry-leading results in pharmaceutical and biotech marketing and sales to Idorsia. She served as CSL Behring's Vice President, Global Commercial Strategy for Thrombosis and Hemostasis; Shire Pharmaceuticals' Executive Vice President Head of US Hematology; Johnson and Johnson's Vice President US Sales and Marketing of the Cardiovascular and Institutional Business and other senior roles at GlaxoSmithKline and AstraZeneca. Working across all stages of the product lifecycle from preclinical to launch to patent expiration and across therapeutic categories, she has forged a reputation for bringing successful products to market: while leading sales and marketing efforts for first-in-class Xa inhibitor Xarelto at J&J, Patty launched six indications in 18 months and achieved over $2 billion in sales.

Ms. Torr earned a B.S. in Public Health Education from East Carolina University and an MBA in Marketing from St. Joseph's University.

**About Idorsia**
Idorsia Ltd is reaching out for more - We have more ideas, we see more opportunities and we want to help more patients. In order to achieve this, we will develop Idorsia into one of Europe's leading biopharmaceutical companies, with a strong scientific core.

Headquartered in Switzerland - a biotech-hub of Europe - Idorsia is specialized in the discovery and development of small molecules, to transform the horizon of therapeutic options. Idorsia has a broad portfolio of innovative drugs in the pipeline, an experienced team, a fully-functional research center, and a strong balance sheet – the ideal constellation to bringing R&D efforts to business success.

Idorsia was listed on the SIX Swiss Exchange (ticker symbol: IDIA) in June 2017 and has over 800 highly qualified specialists dedicated to realizing our ambitious targets.

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