Media Release
February 4, 2021

Idorsia announces financial results for 2020 – a successful year marked by outstanding clinical data

Allschwil, Switzerland – February 4, 2021
Idorsia Ltd (SIX: IDIA) today announced its financial results for the full year 2020.

Business highlights

- Positive results in the Phase 3 program of daridorexant, demonstrating improved overall sleep and daytime functioning of patients with insomnia
- Daridorexant new drug application (NDA) submitted to the US FDA on January 8, 2021
- Positive results in the Japanese registration program for clazosentan, demonstrating reduction in vasospasm-related morbidity and all-cause mortality
- US commercial operations established, with leadership team in place, and Syneos Health selected as commercialization partner to build the salesforce for the launch of daridorexant in the US
- Neurocrine Biosciences entered into a license agreement for the development and commercialization of Idorsia's novel T-type calcium channel blocker
- Axovan arbitration claim dismissed by Arbitral Tribunal

Financial highlights

- Successful capital increases secured over CHF 865 million of funding to prepare for the launch of daridorexant and to develop our diversified pipeline
- US GAAP operating expenses 2020 at CHF 482 million
- Non-GAAP operating expenses 2020 at CHF 444 million
- **Guidance for 2021:** US GAAP operating expenses of ~ CHF 685 million and non-GAAP operating expenses of ~ CHF 640 million (both measures include inventory build of around CHF 35 million and exclude unforeseen events).

Jean-Paul Clozel, MD and Chief Executive Officer, commented:
"I am very proud of all that has been accomplished at Idorsia in 2020. Despite the global pandemic, the company has made great strides, achieving every one of the ambitious key goals set for 2020 – and much else besides. On the back of the positive registration programs for daridorexant and clazosentan in Japan, we have ramped up the build of our global commercial organization and are moving full steam ahead to our first product launches. The year 2021 is key as we are preparing the launch of our first products, expecting key clinical results for lucerastat and cenerimod, and initiating the Phase 3 program for selatogrel. Now, more than ever, the Idorsia story is one to watch!"

Financial results

US GAAP results in CHF millions, except EPS (CHF) and number of shares (millions)	Full Year		Fourth Quarter	
	2020	2019	2020	2019
Revenues	72	24	6	4
Operating expenses	(482)	(506)	(128)	(131)
Operating income (loss)	(411)	(482)	(123)	(127)
Net income (loss)	(445)	(494)	(137)	(142)
Basic EPS	(3.11)	(3.76)	(0.85)	(1.08)
Basic weighted average number of shares	142.8	131.2	160.8	131.2
Diluted EPS	(3.11)	(3.76)	(0.85)	(1.08)
Diluted weighted average number of shares	142.8	131.2	160.8	131.2

US GAAP revenue of CHF 72 million in 2020 consisted of contract revenue recognized in connection with the collaboration agreements with Neurocrine Biosciences, Inc. (CHF 50 million), Janssen Biotech, Inc. (CHF 11 million), Roche (CHF 6 million), Mochida Pharmaceutical Co., Ltd (CHF 3 million) and Santhera Pharmaceuticals Ltd (CHF 2 million) compared to a revenue of CHF 24 million in 2019.

US GAAP operating expenses in 2020 amounted to CHF 482 million (CHF 506 million in 2019), of which CHF 381 million relates to R&D (CHF 439 million in 2019), which includes a one-off expense of CHF 9 million as explained in the legal update below, and CHF 101 million to SG&A expenses (CHF 68 million in 2019).

US GAAP net loss in 2020 amounted to CHF 445 million compared to CHF 494 million in 2019. The decrease of the net loss was mainly driven by higher contract revenues and lower operating expenses, which was partially offset by higher financial expenses.

The US GAAP net loss resulted in a net loss per share of CHF 3.11 (basic and diluted) in 2020 compared to a net loss per share of CHF 3.76 (basic and diluted) in 2019.

Non-GAAP* measures in CHF millions, except EPS (CHF) and number of shares (millions)	Full Year		Fourth Quarter	
	2020	2019	2020	2019
Revenues	72	24	6	4
Operating expenses	(444)	(470)	(142)	(122)
Operating income (loss)	(372)	(446)	(136)	(118)
Net income (loss)	(392)	(448)	(148)	(121)
Basic EPS	(2.75)	(3.41)	(0.92)	(0.92)
Basic weighted average number of shares	142.8	131.2	160.8	131.2
Diluted EPS	(2.75)	(3.41)	(0.92)	(0.92)
Diluted weighted average number of shares	142.8	131.2	160.8	131.2

** 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 2020 amounted to CHF 392 million: the CHF 53 million difference versus US GAAP net loss was mainly due to depreciation and amortization (CHF 19 million), share-based compensation (CHF 16 million) and a negative non-cash financial result (CHF 18 million).

The non-GAAP net loss resulted in a net loss per share of CHF 2.75 (basic and diluted) in 2020 compared to a net loss per share of CHF 3.41 (basic and diluted) in 2019.

Issuance of new registered shares

In 2020, Idorsia issued in total 34.8 million new registered shares from existing authorized share capital receiving gross proceeds of CHF 865.5 million.

As a result of the capital increases, the share capital of Idorsia registered in the commercial register was increased to CHF 8,322,337.30 divided into 166,446,746 registered shares with a nominal value of CHF 0.05 each.

André C. Muller, Chief Financial Officer, commented:
"Our strengthened balance sheet with 1.2 billion Swiss francs liquidity will take us through to the next inflection points – namely, key clinical data from late-stage assets and the launch of our first products. We will invest during 2021 to ensure commercial success of daridorexant in the US and clazosentan in Japan, both anticipated to launch in 2022, following market authorization. As a result, our spend will increase in 2021, with US GAAP operating expenses of around CHF 685 million and non-GAAP operating expenses of around CHF 640 million, both measures include an inventory build of around CHF 35 million and exclude unforeseen events."

Liquidity and indebtedness

At the end of 2020, Idorsia's liquidity (including cash, cash equivalents, short- and long-term deposits) amounted to CHF 1,200 million.

(in CHF millions)	Dec 31, 2020	Sep 30, 2020	Dec 31, 2019
Liquidity			
Cash and cash equivalents	141	148	263
Short-term deposits	867	668	476
Long-term deposits	192	-	-
Total liquidity*	**1,200**	**816**	**739**
Indebtedness			
Convertible loan	388	386	380
Convertible bond	199	199	199
Other financial debt	-	-	-
Total indebtedness	**587**	**585**	**579**

*rounding differences may occur

Clinical Development

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

In April and July of 2020, Idorsia reported positive results in each of the two pivotal Phase 3 studies of **daridorexant** in patients with insomnia. More details and commentary can be found in the dedicated press releases (first study release), (second study release) and the investor webcasts (first study webcast), (second study webcast) which are available for replay on Idorsia's corporate website. On January 8, 2021, the daridorexant new drug application (NDA) was submitted to the US FDA.

In November of 2020, Idorsia reported positive results in each of the two Japanese registration studies of **clazosentan** in patients with cerebral vasospasm following aneurysmal subarachnoid hemorrhage. More details can be found in the dedicated press release.

In addition to the progress made in 2020, the company is initiating a Phase 2 proof-of-concept study with ACT-539313, a selective orexin 1 receptor antagonist, in binge eating disorder. Preclinical studies have shown that orexins play an important role in compulsive, binge-like feeding behaviors. Furthermore, orexin receptor antagonists have reduced binge-like eating behavior in animal models. Our study will be the first to study orexin 1 receptor antagonism as a new mechanism of action for patients with eating disorders.

The company is also initiating a Phase 1 clinical pharmacology program with a new Immunology compound ACT-777991 resulting from our drug discovery efforts.

Following initial exploratory results with ACT-774312, a CRTH2 receptor antagonist, in nasal polyposis, the company has decided not to pursue this indication further. There were no safety concerns raised, however the company has no further plans for the compound at this time.

Idorsia's clinical development pipeline

Compound	Mechanism of Action	Target Indication	Status
Daridorexant	Dual orexin receptor antagonist	Insomnia	NDA submitted, MAA in preparation
Aprocitentan*	Dual endothelin receptor antagonist	Resistant hypertension management	Phase 3
Clazosentan	Endothelin receptor antagonist	Cerebral vasospasm assoc. with aneurysmal subarachnoid hemorrhage	Japan: Filing in preparation Global: Phase 3
Lucerastat	Glucosylceramide synthase inhibitor	Fabry disease	Phase 3
Selatogrel	$P2Y_{12}$ receptor antagonist	Suspected acute myocardial infarction	Phase 3 in preparation
Cenerimod	$S1P_1$ receptor modulator	Systemic lupus erythematosus	Phase 2b
ACT-539313	Selective orexin 1 receptor antagonist	Binge eating disorder	Phase 2
Sinbaglustat	GBA2/GCS inhibitor	Rare lysosomal storage disorders	Phase 1 complete
ACT-1004-1239	CXCR7 antagonist	Immunology	Phase 1
ACT-1014-6470	-	Immunology	Phase 1
ACT-541478	-	CNS	Phase 1
ACT-777991	-	Immunology	Phase 1

** In collaboration with Janssen Biotech to jointly develop aprocitentan, Janssen Biotech has sole commercialization rights worldwide*

Neurocrine Biosciences has a global license to develop and commercialize ACT-709478, a novel T-type calcium channel blocker. In November 2020, Neurocrine announced it had initiated a Phase 2 study investigating ACT-709478 for the treatment of a rare form of pediatric epilepsy.

Further details including the current status of each project in the pipeline can be found in our clinical development fact sheet.

Human Resources

Idorsia created 92 new positions worldwide in 2020, bringing the total number of employees (permanent, post-doc, and apprentices) to 908 (2019: 816).

Annual Report

Full details on the progress made in 2020 are available in Idorsia's 2020 Annual Report, consisting of the Business Report, Governance Report, Compensation Report, and Financial Report, at www.idorsia.com/annual-report

Legal update on the Axovan arbitration

As a result of the demerger of Idorsia from Actelion, Idorsia holds a license agreement to develop and commercialize clazosentan from a share purchase agreement between Actelion and Axovan sellers.

Axovan sellers and F. Hoffman-La Roche Ltd are entitled to receive milestone up to CHF 92 million (CHF 21 million at filing, CHF 51 million at approval and CHF 20 million sales milestones). These milestones had been reduced following the acquisition in 2020 of claims from some Axovan sellers for a one-time cash consideration of CHF 9 million.

In 2018, the assignee of 65% of former Axovan shareholders (the "Claimants") entered into an arbitration against Actelion claiming that the acquisition of Actelion by Johnson & Johnson and/or the Demerger triggers the accelerated payment of all outstanding milestones mainly relating to clazosentan (the "Claim") plus statutory interest for late payment.

On February 1, 2021, Idorsia was notified in a final award by the arbitral tribunal that the Claim had been dismissed.

In the first half 2020, the company accrued CHF 23 million relating to the Axovan arbitration. Following the final award, this accrual was fully reversed in Q4 2020.

Idorsia remains fully committed to developing and commercializing clazosentan and to paying any milestones, if and when due.

Note to Shareholders

The Annual General Meeting (AGM) of Shareholders to approve the Annual Report of the year ending December 31, 2020 will be held on Wednesday May 12, 2021.

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 23, 2021. Any proposal received after the deadline will be disregarded.

In order to vote at the Annual General Meeting, shareholders must be registered in the company's shareholder register by May 3, 2021 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 2021 Financial Results reporting on April 22, 2021
- Annual General Meeting of Shareholders on May 12, 2021
- Half-Year 2021 Financial Results reporting on July 27, 2021
- Nine-months 2021 Financial Results reporting on October 26, 2021

Notes to the editor

Letter to Shareholders from the Chairman (as published in Idorsia's 2020 Business Report on February 4, 2021)

Dear Shareholders,

It is my great honor to write to you as Chairman of your company for the first time. I am only sorry that we have not yet had the opportunity to interact personally. Our virtual Annual General Meeting was just one of the many disruptions caused by the COVID 19 pandemic – a crisis which has impacted every area of our lives in 2020, bringing misery and hardship to countless millions. In our industry, COVID-19 has upended supply chains and clinical trial timelines, threatening longer waiting times for much-needed treatments.

Despite the devastation all around us, I am very proud and excited to report that Idorsia has not only weathered the storm this year, but gone from strength to strength.

Thanks to the investments made during Idorsia's first few years, our technology platforms are state-of-the-art and scalable. As a result, when the call went out for office-based staff to work from home and for those who need to work on-site to be protected, our workforce was able to transition seamlessly. Our laboratory-based staff willingly turned up every day, motivated to move the company forward.

The resilience and determination of our workforce contributed to an incredibly successful year: the company has delivered on every one of the ambitious goals set by the management and Board at the end of 2019. Jean-Paul Clozel's update on these achievements (see the CEO's letter below) makes for very enjoyable reading. As you reflect on these successes, please bear in mind that it is very unusual for a clinical pipeline to advance so positively. For a young company to have so many innovative compounds – at a late stage of development, and in a wide variety of therapeutic areas – progressing as planned is, in itself, an impressive feat. For two of those compounds to achieve positive results and move towards regulatory submissions, in parallel, is outstanding. Accordingly, as you will see, the Board has recognized this performance through Idorsia's employee reward system.

As Chairman, I am well aware that positive results alone are not enough to ensure sustainable success. With several newcomers to the Board, we have undertaken a critical review of the company to make sure that all the appropriate checks and balances are in place. For me, this is another of the beauties of Idorsia: while the company is only a few years old, it has a rich heritage of over 20 years in pharma. A solid focus on science and quality, together with a strong governance framework, means that the company has sound foundations on which to build its success.

As a Board, we are committed to transparency on the topics that are important to you. This year, we initiated a dialogue to identify and prioritize what matters most to you, and these lines of communication will remain open. I look forward to developing our reporting on these important non-financial measures, in line with the company's development.

One result of the company's willingness to engage is the growth of our analyst base during 2020: 14 analysts are now covering Idorsia, reflecting the great interest in Idorsia's equity story. I personally believe that independent stock research is important in guiding investors and highlighting opportunities for long-term value creation.

Of course, if Idorsia is to become sustainable, we now need to bring our products to market and start generating revenue. The capital market funding we were able to access this year will take us a long way towards this goal. Thank you for your confidence in Idorsia – and stay tuned for the continued success of this dynamic company on the verge of great things!

Sincerely,

Mathieu Simon,
Chairman of the Board

Letter to Shareholders from the CEO (as published in Idorsia's 2020 Business Report on February 4, 2021)

Dear Shareholders,

I want to begin by recognizing that, while Idorsia has had a very successful year, 2020 was extremely difficult for many people. No doubt some of you will have been seriously affected by the COVID-19 pandemic, and to you I extend my sincere sympathy. This year, the public has gained a new appreciation of the efforts of all frontline healthcare workers, and also of the mission that drives everyone in the pharma world – finding medicines to prevent or treat illness. Let us hope that the vaccines developed in record time can help the world return to some semblance of normality. I myself am very proud to be a member of this industry, and to see how our efforts in research are helping patients.

In spite of the pandemic, the company has made great strides this year. Our employees achieved every one of the ambitious key goals set for 2020 – and much else besides. Their performance was simply phenomenal. In the development of new medicines, hard work does not always guarantee good results, which makes it all the more rewarding when our efforts are crowned with success.

Revolutionizing the field of insomnia
Our review of the progress made in 2020 must start with the outstanding results achieved with daridorexant. The Phase 3 registration program demonstrated statistically significant and clinically meaningful improvements in sleep maintenance, sleep onset, total sleep time and daytime functioning, which were sustained over time. Daridorexant was well tolerated, with a favorable safety profile in adult and elderly patients.

Particularly important, as well as efficacy during the night, are the effects seen during the day. While a negative impact on daytime functioning is part of the definition of insomnia, not one of the treatments currently available have rigorously assessed their effect on this key aspect of the condition. Here, in fact, most therapies have a negative impact – especially first thing in the morning, when patients may feel hangover effects of their medication. For the millions of people suffering from insomnia, daridorexant is an absolute gamechanger, whose excellent benefit and safety profile will encourage patients to seek treatment. It is important to recognize that these gratifying results were no accident. They are a direct result of our efforts to design a dual orexin receptor antagonist (DORA) with the properties required to deliver all the benefits of a good night's sleep. For more details, I invite you to read the interview with Martine Clozel on page 30 of the Business Report.

The team responsible worked tirelessly to evaluate the huge amount of data generated and to present the results appropriately for regulatory agencies. A new drug application was submitted to the FDA in January 2021, and the submission to the European authorities will follow shortly. In anticipation of regulatory approval, the commercial team is now working flat out to prepare for the launch.

Perseverance finally paying off
Our efforts to design the perfect DORA began in our labs as long ago as 1998, but our work with clazosentan goes back even further. This year, the Japanese studies of clazosentan for patients suffering cerebral vasospasm following aneurysmal subarachnoid hemorrhage also demonstrated excellent efficacy results, without any unexpected safety findings (for more information on clazosentan, see pages 52 to 57 of the Business Report). I am truly delighted to finally have the evidence that clazosentan can improve outcomes for these patients – often young adults with so much life left to live. These results give the commercial team even more to get their teeth into. The impressive data has renewed the enthusiasm of the whole REACT team to complete the global study of clazosentan as soon as possible, so that patients around the world can benefit.

More to come from the pipeline
Thanks to the development group's speedy response to the COVID-19 pandemic, our ongoing late-stage studies were able to continue, and risk mitigation procedures were agreed with global health authorities.

Recruitment for both the CARE study (cenerimod) and the MODIFY study (lucerastat) was affected, but following some adjustments, in consultation with health authorities, patient enrollment was adapted. Accordingly, we should see the results of CARE and be able to plan for Phase 3 development of cenerimod by the end of 2021, and the results of MODIFY – the Phase 3 registration study for lucerastat – should also be available in the second half of 2021.

In addition, again despite the impact of the pandemic, recruitment for the PRECISION (aprocitentan) study accelerated, with randomization now expected to be completed by mid 2021.

As well as the conclusion of studies, 2021 will also see the initiation of a very exciting study. Under a Special Protocol Assessment (SPA) agreed with the FDA in 2020, a 14,000 patient Phase 3 study will be commenced with selatogrel – a drug-device product for patients with suspected heart attack. The investigation of selatogrel has been designated as a "fast-track" development program, indicating the FDA's interest in this innovative approach. Patients themselves will play a crucial role in the preparations for this study, as they need to be trained to identify symptoms and self-administer treatment. The potential implications for future heart attack patients are enormous, and I look forward to sharing more details in due course.

Taking our innovation to the patient
Work on building our commercial capabilities began in 2019, and we continued to fill key strategic roles on the global team in 2020. We also established our US commercial organization, securing premises and – most importantly – a leadership team. This is a very exciting development for Idorsia, and I am proud to have such an experienced and talented group of professionals on board.

Following the positive results achieved with daridorexant, a tremendous amount of work has been done to prepare for the US launch of this product in 2022 – not least, engaging Syneos Health as the ideal partner to reach the large US primary care market. In addition, after the excellent results with clazosentan, the Japanese team is also gearing up for our first product launch in Japan.

With two product launches in preparation, we have also been building our global supply chain function, to ensure consistent supplies of our innovative medicines to patients. More information on our preparations can be found in the interview with Chief Commercial Officer Simon Jose on page 22 of the Business Report.

Ongoing innovation
We have always had a long-term focus for Idorsia, setting ourselves the goal of becoming a fully-fledged biopharmaceutical company, innovating from bench to bedside. As our late stage pipeline starts to bear fruit, it is essential that we keep the pipeline supplied with fresh innovation for sustainable success. As our Chairman emphasized, work in our laboratories continued unabated in 2020, and there is much to show for it. Progress was made with our early-stage clinical pipeline, and we advanced a new CNS compound into clinical pharmacology studies. On the preclinical front, four compounds were selected as preclinical candidates. These advances in our discovery efforts made during lockdown are a testament to our researchers' commitment and dedication.

Financing our future
Through a series of financing activities in 2020, we have secured additional funding of more than CHF 865 million. Our strengthened balance sheet with CHF 1.2 billion liquidity will take us through to the next inflection points – namely, key clinical data from late-stage assets and the launch of our first product, daridorexant. As you may be aware, Martine and I have participated in each of the capital increases, believing more than ever in Idorsia and in the value that can be created for patients, employees and shareholders alike.

I am very proud of all that has been accomplished at Idorsia in 2020. 2021 will be an extremely exciting year for our growing company, and I look forward to keeping you updated on our progress throughout the year. I would also like to thank you for your confidence in Idorsia.

Warmest regards,

Jean-Paul Clozel,
Chief Executive Officer

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 a leading biopharmaceutical company, with a strong scientific core.

Headquartered near Basel, Switzerland – a European biotech-hub – Idorsia is specialized in the discovery, development and commercialization of small molecules to transform the horizon of therapeutic options. Idorsia has a broad portfolio of innovative drugs in the pipeline, an experienced team of professionals covering all disciplines from bench to bedside, state-of-the-art facilities, and a strong balance sheet – the ideal constellation to translate R&D efforts into business success.

Idorsia was listed on the SIX Swiss Exchange (ticker symbol: IDIA) in June 2017 and has over 900 highly qualified specialists dedicated to realizing our ambitious targets.

For further information, please contact
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