Media Release
April 22, 2021

Idorsia announces financial results for the first quarter 2021 – substantial progress made across the pipeline – launch preparations well underway

Allschwil, Switzerland – April 22, 2021
Idorsia Ltd (SIX: IDIA) today announced its financial results for the first quarter of 2021.

Business highlights

- Daridorexant new drug application (NDA) submitted to the US FDA in January 2021 and has been accepted for review
- Daridorexant marketing authorisation application (MAA) submitted to the European Medicines Agency (EMA) in March 2021
- Daridorexant MAA submitted to Switzerland's health authority, Swissmedic, in April 2021
- Daridorexant Phase 3 program has concluded – supporting the chronic use of daridorexant in insomnia
- Ponesimod to treat relapsing forms of multiple sclerosis was approved by the US FDA and a positive CHMP opinion was received in March 2021, Idorsia has a revenue-sharing agreement in respect to ponesimod
- Clazosentan NDA submitted to the Japanese Pharmaceuticals and Medical Devices Agency (PMDA) in March 2021
- MODIFY Phase 3 study with lucerastat for Fabry disease fully recruited, results expected in Q4 2021
- PRECISION Phase 3 study with aprocitentan for resistant hypertension fully recruited, results expected mid-2022
- Recruitment of patients into the CARE Phase 2b study with cenerimod for systemic lupus erythematosus completed, results expected in Q4 2021
- Recruitment commenced into Phase 2 study with ACT-539313 for binge eating disorder in March 2021

Financial highlights

- US GAAP operating expenses in Q1 2021 at CHF 129 million
- Non-GAAP operating expenses in Q1 2021 at CHF 121 million
- Guidance for 2021: US GAAP operating expenses below CHF 685 million and non-GAAP operating expenses below 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:
"This first quarter of 2021 is loaded with important progress and milestones; we submitted the marketing registration dossiers for daridorexant to both the FDA, and the EMA and for clazosentan in Japan, and now in April the MAA submission for daridorexant to Switzerland's Swissmedic has also occurred. J&J received the marketing approval for ponesimod in the US and a positive CHMP opinion in the EU which represents patients being able to benefit from a drug discovered by our scientists and the first revenues for Idorsia. We also completed the recruitment of patients into the pivotal trials for lucerastat and aprocitentan, as well as the large Phase 2b study with cenerimod. We are all getting

ready for the launch of our first products and the transformation of Idorsia into a fully-fledged biopharmaceutical company based on science."

Simon Jose, Chief Commercial Officer of Idorsia, commented:
"It's an exciting time at Idorsia and our growing commercial team continues to work with a laser focus on preparing for the successful launches of daridorexant in the US and Europe and clazosentan in Japan. Following the NDA submission for daridorexant, we continue to build our US team and engage with the medical community and other key stakeholders to advance the science and raise awareness about the burden of insomnia on patients' daily lives. The MAA filing for daridorexant in Europe is also an important milestone. We see a significant medical need for new safe and effective treatment options for insomnia in Europe and are ramping up our activities here following the appointment of Jean-Yves Chatelan, President, Region Europe and Canada."

Financial results

US GAAP results **in CHF millions, except EPS (CHF) and number of shares (millions)**	**First Quarter**	
	2021	**2020**
Revenues	7	5
Operating expenses	(129)	(116)
Operating income (loss)	(122)	(111)
Net income (loss)	(105)	(120)
Basic EPS	(0.63)	(0.91)
Basic weighted average number of shares	166.6	131.3
Diluted EPS	(0.63)	(0.91)
Diluted weighted average number of shares	166.6	131.3

US GAAP revenue of CHF 7 million in the first quarter of 2021 consisted of contract revenue recognized in connection with the collaboration agreements with Neurocrine Biosciences, Inc. (CHF 1 million), Janssen Biotech, Inc. (CHF 3 million), Roche (CHF 2 million) and Mochida Pharmaceutical Co., Ltd (CHF 1 million) compared to a revenue of CHF 5 million in the first quarter of 2020.

US GAAP operating expenses in the first quarter of 2021 amounted to CHF 129 million (CHF 116 million in the first quarter of 2020), of which CHF 97 million relates to R&D (CHF 97 million in the first quarter of 2020) and CHF 31 million to SG&A expenses (CHF 19 million in the first quarter of 2020).

US GAAP net loss in the first quarter of 2021 amounted to CHF 105 million compared to CHF 120 million in the first quarter of 2020. The decrease of the net loss was mainly driven by a positive contribution from financial income, which was partially offset by higher operating expenses.

The US GAAP net loss resulted in a net loss per share of CHF 0.63 (basic and diluted) in the first quarter of 2021 compared to a net loss per share of CHF 0.91 (basic and diluted) in the first quarter of 2020.

Non-GAAP* measures in CHF millions, except EPS (CHF) and number of shares (millions)	First Quarter 2021	 2020
Revenues	7	5
Operating expenses	(121)	(106)
Operating income (loss)	(114)	(101)
Net income (loss)	(95)	(102)
Basic EPS	(0.57)	(0.78)
Basic weighted average number of shares	166.6	131.3
Diluted EPS	(0.57)	(0.78)
Diluted weighted average number of shares	166.6	131.3

** 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 the first quarter of 2021 amounted to CHF 95 million: the CHF 10 million difference versus US GAAP net loss was mainly due to depreciation and amortization (CHF 4 million), share-based compensation (CHF 4 million) and a negative non-cash financial result (CHF 2 million).

The non-GAAP net loss resulted in a net loss per share of CHF 0.57 (basic and diluted) in the first quarter of 2021 compared to a net loss per share of CHF 0.78 (basic and diluted) in the first quarter of 2020.

André C. Muller, Chief Financial Officer, commented:
"The whole company is working with a very cost-conscious attitude. We've hit several important milestones in the first quarter and I'm happy to report that all this has been achieved on a slightly lower spend than initially expected. As a result, we now expect US GAAP operating expenses below CHF 685 million and non-GAAP operating expenses below CHF 640 million for the full year 2021, both measures include an inventory build of around CHF 35 million and exclude unforeseen events."

Liquidity and indebtedness

At the end of the first quarter of 2021, Idorsia's liquidity (including cash, cash equivalents, short- and long-term deposits) amounted to CHF 1,065 million.

(in CHF millions)	Mar 31, 2021	Dec 31, 2020	Mar 31, 2020
Liquidity			
Cash and cash equivalents	128	141	95
Short-term deposits	741	867	357
Long-term deposits	196	192	180
Total liquidity*	**1,065**	**1,200**	**632**
Indebtedness			
Convertible loan	390	388	382
Convertible bond	199	199	199
Other financial debt	-	-	-
Total indebtedness	**589**	**587**	**581**

*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. The Phase 3 registration program demonstrated efficacy of daridorexant on objective and subjective sleep parameters, and an improvement in daytime functioning, while maintaining a favorable safety profile. 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. This week, the final results of the 40-week extension study with daridorexant became available. The study collected information on the safety of long-term treatment as well as allowing an exploratory analysis of the maintenance of efficacy. There were no new emerging safety findings. Moreover, the efficacy on sleep and daytime functioning appeared to be maintained over the longer treatment duration. The Phase 3 program is therefore concluded.

A New Drug Application (NDA) was submitted to the US FDA on January 8, 2021 and a Marketing Authorization Application (MAA) to the European Union EMA on March 2, 2021 and to Switzerland health authority, Swissmedic, on April 20, 2021. Should approval be received, the company anticipates launch in the US in the first half of 2022, followed by other regions thereafter.

Guy Braunstein, MD and Head of Global Clinical Development of Idorsia, commented:
"I'm pleased to report that the Phase 3 development program with daridorexant has been successfully concluded. The final analyses of the 40-week extension study with daridorexant are aligned to the interim results submitted to the health authorities. The totality of the data supports the chronic use of daridorexant in insomnia."

In November of 2020, Idorsia reported positive results in each of the two Japanese registration studies of **clazosentan** assessing the efficacy and safety of clazosentan in reducing vasospasm-related morbidity and all-cause mortality in patients following aneurysmal subarachnoid hemorrhage. More details can be found in the dedicated press release. A New Drug Application (NDA) to the Japanese Pharmaceuticals and Medical Devices Agency (PMDA) for clazosentan was submitted on March 1, 2021. Recruitment into the global REACT study of clazosentan has been impacted by the coronavirus pandemic but is steadily progressing.

The MODIFY Phase 3 study of **lucerastat** in Fabry disease was fully recruited in February 2021, with 118 patients in the Phase 3 study. Results are expected in the fourth quarter of this year, should all patients continue into the open label extension study.

Full recruitment has also just been achieved for PRECISION, a Phase 3 study to demonstrate the antihypertensive effect of **aprocitentan** when added to standard of care in patients with resistant hypertension, with 730 patients randomized. This 12-month study should deliver results in mid-2022.

The CARE study, a large Phase 2b multiple-dose, efficacy and safety study with **cenerimod**, for the treatment of systemic lupus erythematosus completed randomization at the end of February 2021, with 427 patients enrolled. The results are targeted for the fourth quarter of 2021.

A Phase 2 proof-of-concept study with **ACT-539313**, a selective orexin 1 receptor antagonist, in binge eating disorder has begun recruitment. Binge eating disorder is the most common eating disorder characterized by repeated episodes of eating unusually large portions of food in a short period of time (within any 2-hour period). It is associated with a sense of lack of control over what is being eaten and

there is often an absence of pleasure in what has been eaten. Preclinical studies have shown that orexins play an important role in driving compulsive binge-like consumption and that orexin receptor antagonists have reduced binge-like eating behavior in animal models. This is the first study of orexin 1 receptor antagonism as a new mechanism of action for patients with binge eating disorder.

Idorsia's clinical development pipeline

Compound	Mechanism of Action	Target Indication	Status
Daridorexant	Dual orexin receptor antagonist	Insomnia	Under review with FDA/EMA
Aprocitentan*	Dual endothelin receptor antagonist	Resistant hypertension management	Phase 3 recruitment complete
Clazosentan	Endothelin receptor antagonist	Cerebral vasospasm assoc. with aneurysmal subarachnoid hemorrhage	Japan: NDA submitted Global: Phase 3
Lucerastat	Glucosylceramide synthase inhibitor	Fabry disease	Phase 3 recruitment complete
Selatogrel	$P2Y_{12}$ receptor antagonist	Suspected acute myocardial infarction	Phase 3 in preparation
Cenerimod	$S1P_1$ receptor modulator	Systemic lupus erythematosus	Phase 2b recruitment complete
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.

Collaboration update

In 2017, Idorsia entered into a research collaboration with Roche in the field of cancer immunotherapy. In the first quarter of 2021, the agreement was terminated. Idorsia continues drug discovery efforts on this promising molecular target.

About the Revenue Sharing Agreement for ponesimod

Idorsia and Actelion Pharmaceuticals Ltd, one of the Janssen Pharmaceutical Companies of Johnson & Johnson, have entered into a revenue-sharing agreement in respect to ponesimod. Under the terms of the revenue-sharing agreement, Idorsia is entitled to receive quarterly payments of 8% of the net sales of ponesimod products from Actelion.

Annual General Meeting – 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.

The notice was published in the Swiss Official Gazette of Commerce (Schweizerisches Handelsamtsblatt) on April 6, 2021, distributed to Shareholders by post on April 16, 2021, and is available, together with the Company's Annual Report and Compensation Report, on www.idorsia.com/agm.

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

- 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
- Full-Year 2021 Financial Results reporting on February 8, 2022

Notes to the editor

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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