Media Release
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Ad hoc announcement pursuant to Art. 53 LR

Idorsia announces financial results for the third quarter 2021 – company progressing and launch preparations in key markets well underway

Allschwil, Switzerland – October 26, 2021
Idorsia Ltd (SIX: IDIA) today announced its financial results for the third quarter of 2021.

Business highlights

- Five Idorsia affiliates in key European markets (France, Germany, Italy, Spain, UK) established
- Daridorexant for the treatment of insomnia under review with US FDA (Food and Drug Administration), EMA (European Medicines Agency), Swissmedic, and Health Canada
- Clazosentan NDA for the treatment of cerebral vasospasm post aneurysmal subarachnoid hemorrhage (aSAH) under review with the Japanese Pharmaceuticals and Medical Devices Agency (PMDA)
- "SOS-AMI" Phase 3 registration study with selatogrel in suspected acute myocardial infarction (AMI) is recruiting
- Results for CARE Phase 2b study with cenerimod for systemic lupus erythematosus expected in Q4 2021
- Decision on the lucerastat for Fabry disease program awaiting interim analysis of the open-label extension study – expected by year-end
- Issuance of CHF 600 million senior unsecured convertible bonds to fund the development of the company into a leading biopharmaceutical company

Financial highlights

- US GAAP operating expenses Q3 2021 at CHF 150 million
- Non-GAAP operating expenses Q3 2021 at CHF 139 million
- Guidance for 2021: US GAAP operating expenses below CHF 665 million and non-GAAP operating expenses below CHF 620 million (both measures include inventory build of around CHF 35 million and exclude unforeseen events)

Jean-Paul Clozel, MD and Chief Executive Officer, commented:
"We are steadily advancing toward becoming a leading biopharmaceutical company. For this, we have set ourselves ambitious goals, one of which is to deliver at least three products to the market. Launch preparations for daridorexant, our most valuable asset to date, and clazosentan in Japan, are well under way. We have made a big step towards having a world class commercial organization by setting up affiliates in key markets at a fast pace. In terms of our potential new therapies, we are developing our diversified and balanced clinical portfolio with a scientific, data-driven approach. In summary, we are ready for our important product launches, we are making considerable strides across the entire company, and I am excited about our future."

Simon Jose, Chief Commercial Officer of Idorsia, commented:
"We continue to focus on preparing for our first expected launches next year with daridorexant in the US and Europe and clazosentan in Japan. I'm delighted with the caliber of the commercial teams we are building in the affiliates and the ambitious launch plans we have developed for these innovative assets. In the US, our medical and market access teams are engaging with key customers to introduce Idorsia and discuss the significant patient needs in the insomnia market. In Europe, our new General Managers are establishing local affiliates in the top 5 markets and bringing on board their lead teams. In Japan, our medical team is engaging with experts in aSAH who are anticipating the first new pharmacological therapy for this life-threatening condition in over 20 years. I look forward to sharing more about our launch plans in the coming months."

Financial results

US GAAP results in CHF millions, except EPS (CHF) and number of shares (millions)	Nine Months		Third Quarter	
	2021	2020	2021	2020
Revenues	30	66	17	8
Operating expenses	(415)	(354)	(150)	(118)
Operating income (loss)	(385)	(288)	(133)	(110)
Net income (loss)	(383)	(308)	(140)	(118)
Basic EPS	(2.29)	(2.25)	(0.83)	(0.83)
Basic weighted average number of shares	167.0	136.8	167.3	142.6
Diluted EPS	(2.29)	(2.25)	(0.83)	(0.83)
Diluted weighted average number of shares	167.0	136.8	167.3	142.6

US GAAP revenue of CHF 30 million in the first nine months of 2021 consisted of contract revenue recognized in connection with the collaboration agreements with Neurocrine Biosciences, Inc. (CHF 3 million), Janssen Biotech, Inc. (CHF 8 million), Roche (CHF 4 million), Mochida Pharmaceutical Co., Ltd (CHF 4 million), settlement of exchangeable note with Santhera (CHF 12 million) and revenue share from J&J (CHF 0.4 million), compared to a revenue of CHF 66 million in the first nine months of 2020.

US GAAP operating expenses in the first nine months of 2021 amounted to CHF 415 million (CHF 354 million in the first nine months of 2020), of which CHF 288 million relates to R&D (CHF 290 million in the first nine months of 2020) and CHF 127 million to SG&A expenses (CHF 64 million in the first nine months of 2020).

US GAAP net loss in the first nine months of 2021 amounted to CHF 383 million compared to CHF 308 million in the first nine months of 2020. The increase of the net loss was mainly driven by lower contract revenues as well as higher operating expenses, mainly in the commercial functions, which was partially offset by a positive contribution from financial income.

The US GAAP net loss resulted in a net loss per share of CHF 2.29 (basic and diluted) in the first nine months of 2021 compared to a net loss per share of CHF 2.25 (basic and diluted) in the first nine months of 2020.

Non-GAAP* measures in CHF millions, except EPS (CHF) and number of shares (millions)	Nine Months 2021	 2020	Third Quarter 2021	 2020
Revenues	30	66	17	8
Operating expenses	(388)	(302)	(139)	(109)
Operating income (loss)	(357)	(236)	(123)	(102)
Net income (loss)	(347)	(245)	(124)	(107)
Basic EPS	(2.08)	(1.79)	(0.74)	(0.75)
Basic weighted average number of shares	167.0	136.8	167.3	142.6
Diluted EPS	(2.08)	(1.79)	(0.74)	(0.75)
Diluted weighted average number of shares	167.0	136.8	167.3	142.6

** 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 nine months of 2021 amounted to CHF 347 million: the CHF 36 million difference versus US GAAP net loss was mainly due to depreciation and amortization (CHF 13 million), share-based compensation (CHF 13 million) and a negative non-cash financial result (CHF 10 million).

The non-GAAP net loss resulted in a net loss per share of CHF 2.08 (basic and diluted) in the first nine months of 2021 compared to a net loss per share of CHF 1.79 (basic and diluted) in the first nine months of 2020.

André C. Muller, Chief Financial Officer, commented:
"With the cash raise of CHF 600 million through the issuance of a 7-year convertible bond, we will enter 2022 with a strong balance sheet to fund the launches of daridorexant in the US and some European markets and clazosentan in Japan. Making a success of these treatments in these territories is the best way for us to reach sustainable profitability in the mid-term. For the full year 2021 we now expect US GAAP operating expenses below CHF 665 million and non-GAAP operating expenses below CHF 620 million, both measures include an inventory build of around CHF 35 million and exclude unforeseen events."

Issuance of senior unsecured convertible bonds

On July 28, 2021, Idorsia placed CHF 600 million senior unsecured convertible bonds (the "Bonds"), due 2028. The Bonds have a maturity of 7 years and are convertible into 19.0 million newly issued registered shares of Idorsia, sourced from the existing listed conditional share capital, on or after September 14, 2021.

The Bonds have a coupon of 2.125%, and a conversion price of CHF 31.54, corresponding to a conversion premium of 40% above the reference share price of CHF 22.5250, being the volume weighted average price of a share on SIX between launch and close of trading on July 28, 2021.

Liquidity and indebtedness

At the end of the first nine months of 2021, Idorsia's liquidity (including cash, cash equivalents, short- and long-term deposits) amounted to CHF 1,399 million.

(in CHF millions)	Sep 30, 2021	Jun 30, 2021	Dec 31, 2020
Liquidity			
Cash and cash equivalents	122	164	141
Short-term deposits	1,117	763	867
Long-term deposits	160	-	192
Total liquidity*	**1,399**	**927**	**1,200**
Indebtedness			
Convertible loan	394	392	388
Convertible bond	794	199	199
Other financial debt	-	-	-
Total indebtedness	**1,188**	**592**	**587**

*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. A New Drug Application (NDA) for daridorexant 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, to Switzerland's health authority, Swissmedic, on April 20, 2021, and to Health Canada on August 25, 2021, where it has been formally accepted for review. Should approval be received, the company anticipates launch in the US in the second quarter of 2022, followed by other regions thereafter.

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 aSAH. More details can be found in the dedicated press release. A New Drug Application (NDA) to the Japanese PMDA for clazosentan was submitted on March 1, 2021. Due to the intensive care setting for the study, recruitment into the global Phase 3 study of clazosentan (REACT) has been impacted by the coronavirus pandemic but is steadily progressing. The study is enrolling approximately 400 patients with a high risk of developing cerebral vasospasm and delayed cerebral ischemia. The study is expected to conclude around the end of 2022.

In October 2021, Idorsia reported that MODIFY, the Phase 3 study of **lucerastat** for adult patients with Fabry disease did not meet the primary endpoint. Lucerastat was well tolerated and a substantial and consistent reduction of plasma Gb3 was observed, confirming the pharmacological activity of lucerastat. Despite this biological effect, the decrease in neuropathic pain observed in both treatment groups did not differ statistically after six months of treatment, using the patient reported outcome tool. Most patients continued into the open-label extension study and a decision on the future of the program will be determined once the interim data is available, expected before the end of the year.

Jean-Paul Clozel, MD and Chief Executive Officer, commented:
"We are taking an in depth look at the results of MODIFY to fully understand the data and some observations made in the six-month double-blind placebo-controlled treatment period. Together with the interim analysis of the open-label extension study, we will be able to determine the potential long-term benefits to patients and make decisions on how to proceed."

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

In June 2021, Idorsia announced the initiation of a Phase 3 registration study "SOS-AMI" to evaluate the efficacy and safety of self-administered subcutaneous **selatogrel**, Idorsia's $P2Y_{12}$ receptor antagonist, in suspected acute myocardial infarction (AMI). More details and commentary can be found in the dedicated press release and the investor webcast.

The CARE study, a large Phase 2b multiple-dose, efficacy and safety study evaluating **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 is recruiting. This is the first study evaluating orexin 1 receptor antagonism as a new mechanism of action for patients with binge eating disorder. Results of the study are expected around the middle of 2022.

The company has closed a natural history study called "RETRIEVE" which collected disease information from pediatric patients with early onset of rare lysosomal storage disorders (LSDs). The company is considering development options for **sinbaglustat**.

Idorsia's clinical development pipeline

Compound	Mechanism of Action	Target Indication	Status
Daridorexant	Dual orexin receptor antagonist	Insomnia	Under review with health authorities
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 primary endpoint not met Awaiting interim analysis of OLE**
Selatogrel	$P2Y_{12}$ receptor antagonist	Suspected acute myocardial infarction	Phase 3
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-777991	-	Immunology	Phase 1

** In collaboration with Janssen Biotech to jointly develop aprocitentan, Janssen Biotech has sole commercialization rights worldwide*
*** Open-label extension study*

Neurocrine Biosciences has a global license to develop and commercialize ACT-709478 (NBI-827104), Idorsia's novel T-type calcium channel blocker. ACT-709478 is currently investigated in two Phase 2 studies for the treatment of a rare form of pediatric epilepsy and essential tremor.

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

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.

Nine-month financial report

A full financial update is available in Idorsia's 2021 Nine-Month Financial Report, at https://www.idorsia.com/investors/financial-information/financial-archive.

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

- Full-Year 2021 Financial Results reporting on February 8, 2022
- Annual General Meeting of Shareholders on April 14, 2022
- First Quarter 2022 Financial Results reporting on April 26, 2022
- Half-Year 2022 Financial Results reporting on July 26, 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 1,000 highly qualified specialists dedicated to realizing our ambitious targets.

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