Media Release
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Ad hoc announcement pursuant to Art. 53 LR

Idorsia announces financial results for the first half 2021 – Building momentum towards becoming a fully-fledged biopharmaceutical company

Allschwil, Switzerland – July 27, 2021
Idorsia Ltd (SIX: IDIA) today announced its financial results for the first half of 2021.

Business highlights

- Daridorexant for the treatment of insomnia under review with US FDA, EMA, and Swissmedic
- Nine scientific presentations for daridorexant shared at SLEEP 2021
- Ponesimod to treat relapsing forms of multiple sclerosis was approved by the US FDA and the European Commission, Idorsia receives first income from the revenue-sharing agreement in respect to ponesimod
- Clazosentan NDA for the treatment of cerebral vasospasm post aneurysmal subarachnoid hemorrhage (aSAH) submitted to the Japanese Pharmaceuticals and Medical Devices Agency (PMDA) in March 2021
- "SOS-AMI" Phase 3 registration study with selatogrel in suspected acute myocardial infarction (AMI) initiated in June 2021
- Phase 2 study with ACT-539313 for binge eating disorder initiated in March 2021
- Results for MODIFY Phase 3 study with lucerastat for Fabry disease expected in Q4 2021
- Results for PRECISION Phase 3 study with aprocitentan for resistant hypertension expected mid-2022
- Results for CARE Phase 2b study with cenerimod for systemic lupus erythematosus expected in Q4 2021

Financial highlights

- US GAAP operating expenses HY 2021 at CHF 265 million
- Non-GAAP operating expenses HY 2021 at CHF 248 million
- Guidance for 2021: US GAAP operating expenses around CHF 665 million and non-GAAP operating expenses around 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:
"The company is making great progress with delivering on the strategic priorities we defined on Day 1 of Idorsia. Now, just 4 years after establishing the company, we are on the verge of transformation into the fully-fledged biopharmaceutical company we intended to be. The regulatory review and the commercial preparation for daridorexant are progressing well and the team keeps its focus on this important asset. I'm also particularly proud of our newest advancement in the pipeline as selatogrel entered Phase 3 being evaluated as an emergency intervention for suspected AMI at the onset of symptoms. Yet another exciting innovation to watch for Idorsia."

Simon Jose, Chief Commercial Officer of Idorsia, commented:
"We are progressing very well with our preparations for the anticipated approval and launch of daridorexant. In the US, our new medical team is starting to engage with key experts and the recruitment of our market access team is nearing completion. In Europe, we have made great progress in building our commercial footprint and have appointed General Managers in the major markets. In the second half we will increase our engagement with key stakeholders and begin to raise awareness of the science and burden of insomnia. Similarly, we are on track with our plans for the expected launch of clazosentan in Japan – where the incidence of aSAH is more than twice as high as in the rest of the world."

Financial results

US GAAP results in CHF millions, except EPS (CHF) and number of shares (millions)	First Half		Second Quarter	
	2021	2020	2021	2020
Revenues	14	58	7	53
Operating expenses	(265)	(236)	(137)	(120)
Operating income (loss)	(252)	(178)	(130)	(67)
Net income (loss)	(243)	(189)	(139)	(69)
Basic EPS	(1.46)	(1.41)	(0.83)	(0.51)
Basic weighted average number of shares	166.9	133.8	167.1	136.4
Diluted EPS	(1.46)	(1.41)	(0.83)	(0.51)
Diluted weighted average number of shares	166.9	133.8	167.1	136.4

US GAAP revenue of CHF 14 million in the first half of 2021 consisted of contract revenue recognized in connection with the collaboration agreements with Neurocrine Biosciences, Inc. (CHF 2 million), Janssen Biotech, Inc. (CHF 5 million), Roche (CHF 4 million), Mochida Pharmaceutical Co., Ltd (CHF 3 million) and revenue share from J&J (CHF 0.4 million) compared to a revenue of CHF 58 million in the first half of 2020.

US GAAP operating expenses in the first half of 2021 amounted to CHF 265 million (CHF 236 million in the first half of 2020), of which CHF 192 million relates to R&D (CHF 197 million in the first half of 2020) and CHF 74 million to SG&A expenses (CHF 40 million in the first half of 2020).

US GAAP net loss in the first half of 2021 amounted to CHF 243 million compared to CHF 189 million in the first half 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 1.46 (basic and diluted) in the first half of 2021 compared to a net loss per share of CHF 1.41 (basic and diluted) in the first half of 2020.

Non-GAAP* measures	First Half		Second Quarter	
in CHF millions, except EPS (CHF) and number of shares (millions)	2021	2020	2021	2020
Revenues	14	58	7	53
Operating expenses	(248)	(193)	(128)	(86)
Operating income (loss)	(234)	(134)	(121)	(33)
Net income (loss)	(223)	(138)	(128)	(36)
Basic EPS	(1.34)	(1.03)	(0.77)	(0.26)
Basic weighted average number of shares	166.9	133.8	167.1	136.4
Diluted EPS	(1.34)	(1.03)	(0.77)	(0.26)
Diluted weighted average number of shares	166.9	133.8	167.1	136.4

** 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 half of 2021 amounted to CHF 223 million: the CHF 20 million difference versus US GAAP net loss was mainly due to depreciation and amortization (CHF 8 million), share-based compensation (CHF 8 million) and a negative non-cash financial result (CHF 4 million).

The non-GAAP net loss resulted in a net loss per share of CHF 1.34 (basic and diluted) in the first half of 2021 compared to a net loss per share of CHF 1.03 (basic and diluted) in the first half of 2020.

André C. Muller, Chief Financial Officer, commented:

"I'm pleased to now see a regular income stream, albeit modest, with the revenue-sharing agreement relating to ponesimod, which was launched by J&J in the second quarter following its approval for relapsing forms of multiple sclerosis in the US and EU. With regulatory discussions for daridorexant in the US and Europe, and clazosentan in Japan, progressing well, the investment into our global commercial infrastructure and launch preparations will gradually increase over the next quarters leading to US GAAP operating expenses for the full year 2021 around CHF 665 million and non-GAAP operating expenses around CHF 620 million, both measures include an inventory build of around CHF 35 million and exclude unforeseen events."

Liquidity and indebtedness

At the end of the first half of 2021, Idorsia's liquidity (including cash, cash equivalents, short- and long-term deposits) amounted to CHF 927 million.

(in CHF millions)	Jun 30, 2021	Mar 31, 2021	Dec 31, 2020
Liquidity			
Cash and cash equivalents	164	128	141
Short-term deposits	763	741	867
Long-term deposits	-	196	192
Total liquidity*	**927**	**1,065**	**1,200**
Indebtedness			
Convertible loan	392	390	388
Convertible bond	199	199	199
Other financial debt	-	-	-
Total indebtedness	**592**	**589**	**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 and to Switzerland health authority, Swissmedic, on April 20, 2021. 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 (high-risk prevention group). The recruitment of patients with confirmed cerebral vasospasm (early treatment group) has been very low, so following a recommendation received by the study Independent Data Monitoring Committee (IDMC), the company has decided to discontinue this relatively small treatment group. Results continue to be expected in the second half of 2022.

The MODIFY Phase 3 study of Idorsia's oral substrate reduction therapy **lucerastat** in patients with Fabry disease irrespective of mutation type was fully recruited in February 2021, with 118 patients. Results continue to be expected in the fourth quarter of this year.

Full recruitment has also 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.

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.

Based on initial clinical pharmacology investigation with **ACT-541478**, the company has decided not to pursue the development of this CNS compound further.

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 now considering development options for **sinbaglustat**.

Neurocrine Biosciences recently initiated a second Phase 2 study with **ACT-709478** (NBI-827104), Idorsia's novel T-type calcium channel blocker, in patients with essential tremor, in addition to the ongoing Phase 2 study in a rare form of pediatric epilepsy.

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 recruitment complete
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*

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.

Half-year financial report

A full financial update is available in Idorsia's 2021 Half Year Financial Report, at www.idorsia.com/investors/corporate-reports.

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

- Nine-months 2021 Financial Results reporting on October 26, 2021
- Full-Year 2021 Financial Results reporting on February 8, 2022
- First Quarter 2022 Financial Results reporting on April 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.

For further information, please contact

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