Media Release
October 27, 2020

Idorsia announces financial results for the first nine months of 2020

Allschwil, Switzerland – October 27, 2020
Idorsia Ltd (SIX: IDIA) today announced its financial results for the first nine months of 2020.

Business highlights

- Positive results in the second pivotal Phase 3 study of daridorexant
- Results from the first pivotal Phase 3 study of daridorexant presented at SLEEP 2020
- Daridorexant dose response in Japanese patients with insomnia confirmed
- Commercial preparation advancing:
  - US commercial operations and leadership team established
  - Syneos Health appointed as commercialization partner to launch daridorexant in the US
- Issuance of 23.8 million new shares receiving gross proceeds of CHF 535.5 million – increased liquidity will be used to prepare for the launch of daridorexant and further fund Idorsia's diversified pipeline

Financial highlights

- US GAAP operating expenses 9M 2020 at CHF 354 million
- Non-GAAP operating expenses 9M 2020 at CHF 302 million
- Updated guidance for 2020: US GAAP operating expenses around CHF 500 million and non-GAAP operating expenses around CHF 460 million (both measures exclude unforeseen events, potential milestone payments and any potential payments related to the Axovan arbitration)

Jean-Paul Clozel, MD and Chief Executive Officer, commented:
"In the third quarter, we built on the outstanding results we saw with the first pivotal study of daridorexant for insomnia. We reported the second positive pivotal study, we confirmed that the pooled data from the respective placebo and 25 mg dose arms supports the safety and efficacy, the interim analysis of the long-term safety and efficacy did not uncover new safety findings while showing that the efficacy is maintained, and we completed the clinical pharmacology package. This means that we have collected the required data for filing the New Drug Application. Following our initial discussions with the FDA we remain on target to file around the end of 2020. We are also making great progress with the market preparation and the most recent round of financing ensures that – subject to approval – we can bring our first product to patients, as well as seeing the results of registration studies from other late-stage assets. We now continue to deliver on the high expectations that we set for ourselves."

Financial results

US GAAP results in CHF millions, except EPS (CHF) and number of shares (millions)	Nine Months		Third Quarter	
	2020	2019	2020	2019
Revenues	66	20	8	7
Operating expenses	(354)	(375)	(118)	(123)
Operating income (loss)	(288)	(355)	(110)	(116)
Net income (loss)	(308)	(352)	(118)	(120)
Basic EPS	(2.25)	(2.68)	(0.83)	(0.91)
Basic weighted average number of shares	136.8	131.2	142.6	131.2
Diluted EPS	(2.25)	(2.68)	(0.83)	(0.91)
Diluted weighted average number of shares	136.8	131.2	142.6	131.2

US GAAP revenue of CHF 66 million in the first nine months of 2020 consisted of contract revenue recognized in connection with the collaboration agreements with Neurocrine Biosciences, Inc. (CHF 49 million), Janssen Biotech, Inc. (CHF 8 million), Roche (CHF 4 million), Mochida Pharmaceutical Co., Ltd (CHF 3 million) and Santhera Pharmaceuticals Ltd (CHF 2 million) – see further update with regards to Santhera below – compared to a revenue of CHF 20 million in the first nine months of 2019.

US GAAP operating expenses in the first nine months of 2020 amounted to CHF 354 million (CHF 375 million in 9M 2019), of which CHF 290 million relates to R&D (CHF 327 million in 9M 2019), which includes a one-off expense of CHF 32 million as explained in the legal update in the HY 2020 financial reporting and repeated below, and CHF 64 million to SG&A expenses (CHF 47 million in 9M 2019).

US GAAP net loss in the first nine months of 2020 amounted to CHF 308 million compared to CHF 352 million in the first nine months of 2019. The decrease of the net loss was mainly driven by higher contract revenues and lower operating expenses.

The US GAAP net loss resulted in a net loss per share of CHF 2.25 (basic and diluted) in the first nine months of 2020 compared to a net loss per share of CHF 2.68 (basic and diluted) in the first nine months of 2019.

Non-GAAP* measures in CHF millions, except EPS (CHF) and number of shares (millions)	Nine Months		Third Quarter	
	2020	2019	2020	2019
Revenues	66	20	8	7
Operating expenses	(302)	(347)	(109)	(113)
Operating income (loss)	(236)	(328)	(102)	(107)
Net income (loss)	(245)	(326)	(107)	(104)
Basic EPS	(1.79)	(2.49)	(0.75)	(0.79)
Basic weighted average number of shares	136.8	131.2	142.6	131.2
Diluted EPS	(1.79)	(2.49)	(0.75)	(0.79)
Diluted weighted average number of shares	136.8	131.2	142.6	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 the first nine months of 2020 amounted to CHF 245 million: the CHF 63 million difference versus US GAAP net loss was mainly due to depreciation and amortization (CHF 14 million),

share-based compensation (CHF 16 million), an accrual in relation to the ongoing arbitration as explained below (CHF 23 million) and a negative non-cash financial result (CHF 11 million).

The non-GAAP net loss resulted in a net loss per share of CHF 1.79 (basic and diluted) in the first nine months of 2020 compared to a net loss per share of CHF 2.49 (basic and diluted) in the first nine months of 2019.

André C. Muller, Chief Financial Officer, commented:
"We confirm the outlook issued ahead of the capital increase on October 8, 2020, anticipating non-GAAP operating expenses for 2020 of 460 million Swiss francs – excluding unforeseen events, potential milestone payments and any payments related to the Axovan arbitration. The lower spend was mainly caused by COVID-19 which initially impacted the recruitment pace in our late-stage pipeline studies. Furthermore, the capital increase was a great success broadening our shareholder base as well as extending our cash runway well into the launch of daridorexant. Moving forward, other financing instruments should become available to fund the company until break-even."

Issuance of new registered shares

On October 23, 2020 Idorsia issued 23.8 million new registered shares from existing authorized share capital at CHF 22.50 per new share receiving gross proceeds of CHF 535.5 million through an at-market rights offering.

As a result of the capital increase, the share capital of Idorsia registered in the commercial register was increased from CHF 7,132,337.30 to CHF 8,322,337.30 divided into 166,446,746 registered shares with a nominal value of CHF 0.05 each.

Liquidity and indebtedness

At the end of the first nine months of 2020, Idorsia's liquidity (including cash, cash equivalents, short- and long-term deposits) amounted to CHF 816 million.

(in CHF millions)	Sep 30, 2020	Jun 30, 2020	Dec 31, 2019
Liquidity			
Cash and cash equivalents	148	381	263
Short-term deposits	668	348	476
Long-term deposits	-	180	-
Total liquidity*	**816**	**908**	**739**
Indebtedness			
Convertible loan	386	384	380
Convertible bond	199	199	199
Other financial debt	-	-	-
Total indebtedness	**585**	**583**	**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.

Compound	Mechanism of Action	Target Indication	Status
Daridorexant	Dual orexin receptor antagonist	Insomnia	Filing in preparation
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
Selatogrel	$P2Y_{12}$ receptor antagonist	Suspected acute myocardial infarction	Phase 3 in preparation
Cenerimod	$S1P_1$ receptor modulator	Systemic lupus erythematosus	Phase 2
ACT-774312	CRTH2 receptor antagonist	Nasal polyposis	Phase 2
ACT-539313	Selective orexin 1 receptor antagonist	Psychiatric disorders	Phase 2 in preparation
Sinbaglustat	GBA2/GCS inhibitor	Rare lysosomal storage disorders	Phase 1 complete
ACT-1004-1239	-	Immunology / Cancer immunotherapy	Phase 1
ACT-1014-6470	-	Immunology	Phase 1
ACT-541478	-	CNS	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 our ACT-709478, a novel T-type calcium channel blocker, for the treatment of a rare form of pediatric epilepsy. In May 2020, Neurocrine announced plans to initiate a Phase 2 study for ACT-709478 in the second half of 2020.

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

Nine-month financial report

A full financial update is available in Idorsia's 2020 Nine-Month Financial Report, at www.idorsia.com/results-day-center.

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 2020 Financial Results reporting on February 4, 2021
- 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

Notes to the editor

Legal update on milestones regarding clazosentan and related ongoing arbitration

Following the demerger from Actelion and the transfer of a share purchase agreement with the former shareholders Axovan Ltd. (the "Axovan vendors"), Idorsia holds a license agreement to develop and commercialize clazosentan.

In 2018, approximately 65% of Axovan vendors (Claimants) entered an arbitration against Actelion claiming that the acquisition of Actelion by J&J and/or the demerger triggers the accelerated payment of all outstanding milestones under the license agreement. These claims are being vigorously contested by Actelion and by Idorsia, which is required pursuant to the demerger agreement to indemnify Actelion in respect of the claims.

In the first half 2020, Idorsia acquired all outstanding future milestone claims from approximately 26% of Axovan vendors at around 30% of their potential nominal value for a one-time payment of CHF 9 million. The company assessed that this transaction with non-claimants is the best estimate to assess all other vendors' claims, resulting in an accrual of CHF 23 million and a total R&D expense CHF 32 million. At this stage, it is difficult to predict the outcome of the ongoing arbitration which is substantially completed. This accrual may or may not cover the outcome of the ongoing arbitration that could result in a payment between CHF 0 and CHF 94 million.

Update on Santhera

Idorsia currently owns 1.7 million shares in Santhera. Idorsia recognized in its financial statements ending 30 September 2020 contract revenue of CHF 2.4 million corresponding to the fair market value of 336,667 Santhera shares, with CHF 10 milllion of an exchangeable note not being recorded as contract revenue since its recoverability will depend on Santhera's ability to raise sufficient cash. For the nine months ended 30 September 2020, Idorsia booked in its financial result an unrealized loss of CHF 6.8 million corresponding to the change in the fair market value of its 1.7 million Santhera shares.

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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The above information contains certain "forward-looking statements", relating to the company's business, which can be identified by the use of forward-looking terminology such as "estimates", "believes", "expects", "may", "are expected to", "will", "will continue", "should", "would be", "seeks", "pending" or "anticipates" or similar expressions, or by discussions of strategy, plans or intentions. Such statements include descriptions of the company's investment and research and development programs and anticipated expenditures in connection therewith, descriptions of new products expected to be introduced by the company and anticipated customer demand for such products and products in the company's existing portfolio. Such statements reflect the current views of the company with respect to future events and are subject to certain risks, uncertainties and assumptions. Many factors could cause the actual results, performance or achievements of the company to be materially different from any future results, performances or achievements that may be expressed or implied by such forward-looking statements. Should one or more of these risks or uncertainties materialize, or should underlying assumptions prove incorrect, actual results may vary materially from those described herein as anticipated, believed, estimated or expected.