Media Release
July 23, 2020

Idorsia announces financial results for the first half 2020

Allschwil, Switzerland – July 23, 2020
Idorsia Ltd (SIX: IDIA) today announced its financial results for the first half of 2020.

Business updates

- Positive results in both pivotal Phase 3 studies of daridorexant with improved overall sleep and daytime functioning of patients with insomnia
- Neurocrine Biosciences exercised option to license Idorsia's novel treatment for rare pediatric epilepsy
- Establishment of Idorsia Pharmaceuticals US Inc. commercial operations and leadership team
- Merger of Vaxxilon Ltd into Idorsia creating a new unit specialized in synthetic carbohydrate vaccines to prevent infections.
- Janssen submitted New Drug Application to the US FDA and European Marketing Authorization Application for ponesimod for treatment of adults with relapsing multiple sclerosis – Idorsia has a revenue-sharing agreement in respect to ponesimod

Financial updates

- Issuance of 11 million new shares receiving gross proceeds of CHF 330 million
- US GAAP operating expenses HY 2020 at CHF 236 million
- Non-GAAP operating expenses HY 2020 at CHF 193 million
- Updated guidance for 2020: US GAAP operating expenses around CHF 530 million and non-GAAP operating expenses around CHF 490 million (both measures exclude unforeseen events, potential milestone payments and any potential award granted in the ongoing arbitration – see legal update below)

Jean-Paul Clozel, MD and Chief Executive Officer, commented:
"We continue to make great progress at Idorsia, a special achievement considering the extraordinary circumstances brought about by the COVID-19 pandemic. Our clinical team, together with the investigation sites, have done an excellent job to maintain the continuity of the ongoing studies, while ensuring quality and patient safety is not compromised. Of course, the highlight for us in the first half of 2020 is the positive results we've seen with daridorexant investigated as a treatment for insomnia. I'm looking forward to sharing the detailed results, particularly the positive effects we saw on daytime functioning, through scientific publication and preparing the regulatory dossier to file with the US FDA around the end of this year. We're now moving rapidly to establish our commercial capabilities to ensure a successful launch."

Financial results

US GAAP results in CHF millions, except EPS (CHF) and number of shares (millions)	First Half 2020	 2019	Second Quarter 2020	 2019
Revenues	58	13	53	7
Operating expenses	(236)	(252)	(120)	(127)
Operating income (loss)	(178)	(239)	(67)	(121)
Net income (loss)	(189)	(232)	(69)	(126)
Basic EPS	(1.41)	(1.77)	(0.51)	(0.96)
Basic weighted average number of shares	133.8	131.1	136.4	131.2
Diluted EPS	(1.41)	(1.77)	(0.51)	(0.96)
Diluted weighted average number of shares	133.8	131.1	136.4	131.2

US GAAP revenue of CHF 58 million in the first half of 2020 consisted of contract revenue recognized in connection with the collaboration agreements with Neurocrine Biosciences, Inc. (CHF 48 million), Janssen Biotech, Inc. (CHF 6 million), Roche (CHF 3 million) and Mochida Pharmaceutical Co., Ltd (CHF 2 million), compared to a revenue of CHF 13 million in the first half of 2019.

US GAAP operating expenses in the first half of 2020 amounted to CHF 236 million (CHF 252 million in HY 2019), of which CHF 197 million relates to R&D (CHF 220 million in HY 2019), which includes a one-off expense of CHF 32 million as explained in the legal update below and CHF 40 million to SG&A expenses (CHF 33 million in HY 2019).

US GAAP net loss in the first half of 2020 amounted to CHF 189 million compared to CHF 232 million in the first half 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 1.41 (basic and diluted) in the first half of 2020 compared to a net loss per share of CHF 1.77 (basic and diluted) in the first half of 2019.

Non-GAAP* measures in CHF millions, except EPS (CHF) and number of shares (millions)	First Half 2020	 2019	Second Quarter 2020	 2019
Revenues	58	13	53	7
Operating expenses	(193)	(234)	(86)	(118)
Operating income (loss)	(134)	(221)	(33)	(111)
Net income (loss)	(138)	(222)	(36)	(115)
Basic EPS	(1.03)	(1.69)	(0.26)	(0.87)
Basic weighted average number of shares	133.8	131.1	136.4	131.2
Diluted EPS	(1.03)	(1.69)	(0.26)	(0.87)
Diluted weighted average number of shares	133.8	131.1	136.4	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 half of 2020 amounted to CHF 138 million: the CHF 51 million difference versus US GAAP net loss was mainly due to depreciation and amortization (CHF 9 million), share-based compensation (CHF 13 million), an accrual in relation to the arbitration (CHF 24 million) and a negative non-cash financial result (CHF 6 million).

The non-GAAP net loss resulted in a net loss per share of CHF 1.03 (basic and diluted) in the first half of 2020 compared to a net loss per share of CHF 1.69 (basic and diluted) in the first half of 2019.

André C. Muller, Chief Financial Officer, commented:
"We currently anticipate non-GAAP operating expenses of 490 million Swiss francs – excluding unforeseen events, potential milestone payments and any payments related to the ongoing arbitration. The lower spend is mainly caused by COVID-19 that impacted the recruitment pace in our late-stage pipeline studies; the good news is that no studies have been stopped. On the back of the first pivotal trial of daridorexant and the collaboration with Neurocrine, the company raised 323 million Swiss francs in an offering of 11 million new shares, narrowing the liquidity gap."

Issuance of new registered shares

On May 22, 2020 Idorsia privately placed 11 million new registered shares of CHF 0.05 par value from existing authorized share capital at CHF 30 per new share receiving gross proceeds of CHF 330 million, by way of an accelerated book-building process.

Liquidity and indebtedness

At the end of the first half of 2020, Idorsia's liquidity (including cash, cash equivalents, short- and long-term deposits) amounted to CHF 908 million.

(in CHF millions)	Jun 30, 2020	Mar 31, 2020	Dec 31, 2019
Liquidity			
Cash and cash equivalents	381	95	263
Short-term deposits	348	357	476
Long-term deposits	180	180	-
Total liquidity*	**908**	**632**	**739**
Indebtedness			
Convertible loan	384	382	380
Convertible bond	199	199	199
Other financial debt	-	-	-
Total indebtedness	**583**	**581**	**579**

*rounding differences may occur

Commercial Operations

In July 2020, Idorsia announced its strategic expansion in the United States by establishing commercial operations. Located in Radnor, PA – a Philadelphia suburb known for its strong healthcare and pharmaceutical environment – Idorsia Pharmaceuticals US Inc. has been established to further develop and embed Idorsia's budding global commercial organization and realize the value of its innovations by ensuring patients can benefit from the company's pioneering therapies. Led by Patricia "Patty" Torr, President and General Manager, who joined Idorsia in March 2020, the US operations has a talented leadership team with deep experience in the biopharmaceutical sector. Read more about Patty and the team on our US website: www.idorsia.us

Idorsia has selected Omnicom Group, a global leader in marketing communications, as the commercial agency of record for daridorexant across most functions and audiences. Omnicom will bring their network of companies together to fulfil the broad marketing communication needs of Idorsia for the launch of daridorexant globally.

Simon Jose, Chief Commercial Officer, commented:
"We are moving at full power following the outstanding results with daridorexant – building our commercial organization in the US, so that we can rapidly advance our launch preparation. The people we have brought on-board each have the diverse experience and innovative mindset needed to capitalize on our science and commercialize such a broad pipeline. We are conscious to remain flexible and nimble in how we commercialize our portfolio, building the core capabilities required to successfully launch our products, while being prepared to partner where we need support to reach the primary care market."

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 2020, Idorsia reported positive results in each of the two pivotal Phase 3 studies of **daridorexant** in patients with insomnia. The program demonstrated efficacy of treatment with daridorexant on objective and subjective sleep parameters, and daytime functioning, with patients reporting no morning sleepiness, and no evidence of rebound or withdrawal symptoms upon treatment discontinuation. More details and commentary can be found in the dedicated press releases (first study), (second study) and the investor webcasts (first study), (second study) which are available for replay on the corporate website.

For **clazosentan** in Japan, the two registration studies completed recruitment in the first half of 2020. We anticipate a slight delay to collecting the data for these studies due to COVID-19, but still expect the results before the end of the year.

In the context of the COVID-19 pandemic, Idorsia's portfolio of late-stage clinical studies has continued to progress, with a primary focus on ensuring the safety and well-being of patients already participating, as well as study integrity and compliance with Good Clinical Practice (GCP) and applicable regulation in the different regions of operations. No late-stage study has been stopped, processes have been adapted where needed, including development of study-specific crisis plans, to minimize the impact of the pandemic situation and maintain the overall conduct of the studies. During the peak of the pandemic, recruitment of new patients into the late-stage studies slowed. As countries and healthcare systems return to somewhat normality, the recruitment is picking back up. In some countries, where the pandemic continues to impact everyday life, recruitment rate is still being affected. This has resulted in extended recruitment times for the ongoing studies, as indicated in April 2020, and the company is taking steps to accelerate recruitment in an attempt to regain some ground lost during peak period of the pandemic.

Idorsia is making progress with its early development pipeline. Based on a non-clinical program and completed Phase 1 studies, Idorsia considers developing **sinbaglustat** in rare lysosomal storage disorders (LSDs). To collect disease information from pediatric patients with early onset of LSDs, the company is conducting a natural history study called "RETRIEVE". In addition, the company initiated a Phase 1 program with a new compound, **ACT-541478**, intended to be developed in CNS disorders.

Clinical Development Pipeline

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 and solely commercialize Idorsia's aprocitentan worldwide.*

Neurocrine Biosciences has a global license to develop and commercialize Idorsia's ACT-709478, a novel T-type calcium channel blocker, for the treatment of a rare form of pediatric epilepsy. A Phase 2 study is planned for the second half of 2020.

Idorsia has the option to license vamorolone from ReveraGen Inc. and has granted to Santhera Holding Ltd the option to sub-license vamorolone worldwide (except Japan and South-Korea) for all indications.

Further details of the pipeline can be found in our clinical development fact sheet.

On May 12, 2020, Idorsia and Neurocrine Biosciences, Inc. announced that, following Investigational New Drug (IND) application acceptance by the US Food and Drug Administration (FDA), Neurocrine Biosciences had exercised its option to license the global rights to Idorsia's ACT-709478. Neurocrine Biosciences plans to initiate a Phase 2 study with this potent, selective, orally active and brain penetrating T-type calcium channel blocker for the treatment of a rare pediatric epilepsy in the second half of 2020.

On June 26, 2020, Vaxxilon Ltd was merged into Idorsia. Vaxxilon Deutschland GmbH – now a 100% subsidiary of Idorsia – is specialized in synthetic carbohydrate vaccines to prevent infections. Idorsia is now advancing vaccines against *Clostridium difficile* and resistant *Klebsiella pneumoniae*.

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 Pharmaceuticals Ltd.

Legal update on milestones regarding clazosentan and related ongoing arbitration

Following the demerger from Actelion and the transfer of a share purchase agreement with 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. These claims are being vigorously contested by Actelion and by Idorsia, that would be liable to pay any successful claims to the Axovan vendors.

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 24 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.

Half-year financial report

A full financial update is available in Idorsia's 2020 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 2020 Financial Results reporting on October 22, 2020
- Full-Year 2020 Financial Results reporting on February 4, 2021
- First Quarter 2021 Financial Results reporting on April 22, 2021

Notes to the editor

About Omnicom Group

Omnicom Group (www.omnicomgroup.com) is a leading global marketing and corporate communications company. Omnicom's branded networks and numerous specialty firms provide advertising, strategic media planning and buying, digital and interactive marketing, direct and promotional marketing, public relations and other specialty communications services to over 5,000 clients in more than 70 countries.

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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