Media Release
February 7, 2019

Idorsia announces financial results for 2018 – outstanding progress made – four late stage assets advanced into Phase 3

Allschwil, Switzerland – February 7, 2019
Idorsia Ltd (SIX: IDIA) today announced its financial results for the full year 2018.

Key Updates

- Advancing pipeline – four Phase 3 programs initiated
- Operational independence – completed demerger activities
- Chief Commercial Officer appointed – creating a global organization
- Strengthened cash position – to continue investment in our diverse pipeline
- US GAAP operating expenses 2018 at CHF 432 million
- Non-GAAP operating expenses 2018 at CHF 399 million, including CHF 15 million milestone payment to ReveraGen, in line with 2018 guidance
- Guidance for 2019: US-GAAP operating expenses of around CHF 570 million and non-GAAP operating expenses of around CHF 530 million (both measures exclude any potential milestone payments).

Jean-Paul Clozel, MD and Chief Executive Officer, commented:
"Idorsia made outstanding progress with all of our strategic priorities in 2018. We advanced our late-stage pipeline, bringing four products into Phase 3 development. On top of that enormous undertaking, we have advanced our early-stage development pipeline and continued our discovery efforts. With the appointment of Simon Jose as Chief Commercial Officer, we have started to build a global commercial organization to realize the full potential of Idorsia's innovative pipeline and make strategic decisions on how to commercialize our broad range of assets."

Financial results

US GAAP results	**Full Year**		**Fourth Quarter**	
in CHF million, except EPS (CHF) and number of shares (million)	**2018**	**2017**	**2018**	**2017**
Revenues	61	158	41	158
Operating expenses	(432)	(166)	(141)	(87)
Operating income (loss)	(371)	(8)	(101)	71
Net income (loss)	(386)	(14)	(108)	68
Basic EPS	(3.10)	(0.13)	(0.83)	0.57
Basic weighted average number of shares	124.8	114.0	131.1	119.1
Diluted EPS	(3.10)	(0.13)	(0.83)	0.43
Diluted weighted average number of shares	124.8	114.0	131.1	157.9

For the Full Year 2018, US GAAP net loss amounted to CHF 386 million compared to CHF 14 million for the period ending December 31, 2017. The increase of the net loss was mainly driven by lower revenues and higher operating costs.

Revenue of CHF 61 million in 2018 related to deferred contract revenue (CHF 26 million) and an option and sublicensing agreement with Santhera (vamorolone, CHF 34 million) whilst revenue in 2017 of CHF 158 million related to the recognized portion of the upfront milestone received from Janssen (aprocitentan).

Operating expenses of CHF 432 million in 2018 represented 12 months of operations (of which CHF 370 million R&D and CHF 61 million G&A expenses) whilst operating expenses of CHF 166 million in 2017 represented 6.5 months of operations since demerger from Actelion on June 15, 2017 (of which CHF 135 million R&D and CHF 31 million G&A expenses).

The US GAAP net loss resulted in a net loss per share of CHF 3.10 (basic and diluted) for the Full Year 2018 compared to a net loss per share of CHF 0.13 (basic and diluted) for the period ending December 31, 2017.

Non-GAAP* measures	**Full Year**		**Fourth Quarter**	
in CHF million, except EPS (CHF) and number of shares (million)	**2018**	**2017**	**2018**	**2017**
Revenues	61	158	41	158
Operating expenses	(399)	(150)	(133)	(79)
Operating income (loss)	(339)	8	(92)	79
Net income (loss)	(340)	5	(91)	77
Basic EPS	(2.72)	0.04	(0.70)	0.65
Basic weighted average number of shares	124.8	114.0	131.1	119.1
Diluted EPS	(2.72)	0.03	(0.70)	0.49
Diluted weighted average number of shares	124.8	139.5	131.1	157.9

** 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.*

For the Full Year 2018, non-GAAP net loss amounted to CHF 340 million: the difference versus US GAAP net loss was mainly due to depreciation and amortization (CHF 20 million), share-based compensation (CHF 13 million) and non-cash financial expenses (CHF 16 million).

Non-GAAP revenue of CHF 61 million comprised CHF 20 million cash received from Santhera and CHF 41 million non-cash consideration (CHF 15 million with 1 million shares of Santhera and CHF 26 million of deferred recognition from upfront payments in connection with the Roche and Janssen collaboration agreements).

Non-GAAP net loss per share amounted to CHF 2.72 (basic and diluted) for the Full Year 2018 compared to a net profit per share of CHF 0.04 (basic) and CHF 0.03 (diluted) for the period ending December 31, 2017.

André C. Muller, Chief Financial Officer, commented:
"In 2018, we completed demerger activities, with all core systems now running independently of Actelion - this is an outstanding achievement in such a short space of time. We also strengthened our cash position so that we can continue to invest in our diverse pipeline of unique assets that offer significant potential to patients and investors alike. Based on the current status and expected progress of the pipeline, excluding unforeseen events and potential milestones payments, Idorsia expects non-GAAP operating expenses for 2019 to be around CHF 530 million."

Liquidity and indebtedness

At the end of 2018, Idorsia's liquidity (including cash, cash equivalents, short- and long-term deposits) amounted to CHF 1,220 million.

(in CHF millions)	Dec 31, 2018	Sep 30, 2018	Dec 31, 2017
Liquidity			
Cash and cash equivalents	799	671	622
Short-term deposits	123	132	218
Long-term deposits	298	548	250
Total liquidity*	**1,220**	**1,351**	**1,091**
Indebtedness			
Convertible loan	372	370	365
Convertible bond	198	198	-
Other financial debt	-	-	-
Total indebtedness	**571**	**569**	**365**

*rounding differences may occur

Clinical Development Pipeline

Idorsia has a diversified and balanced clinical development pipeline covering multiple therapeutic areas, including CNS, cardiovascular and immunological disorders, as well as orphan diseases.

The late-stage pipeline advanced significantly in 2018, with four compounds moving into Phase 3 clinical development. All late-stage trials are now recruiting patients, with data expected to be reported in 2020 and 2021.

Details of the Phase 3 programs can be found in a series of investor webcasts published on our corporate website.

Progress was also made with our early-stage compounds by accumulating information on clinical pharmacology in healthy volunteers or conducting Phase 2 profiling in patients.

Compound	Mechanism of Action	Target Indication	Status
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
ACT-541468	Dual orexin receptor antagonist	Insomnia	Phase 3
Cenerimod	$S1P_1$ receptor modulator	Systemic lupus erythematosus	Phase 2
Selatogrel	P2Y12 receptor antagonist	Acute coronary syndrome	Phase 2
ACT-774312	CRTH2 receptor antagonist	Nasal polyposis	Phase 2
ACT-519276	GBA2/GCS inhibitor	Orphan CNS diseases	Phase 1
ACT-539313	Selective orexin 1 receptor antagonist	Anxiety	Phase 1
ACT-709478	T-type calcium channel blocker	Epilepsy	Phase 1

** In collaboration with Janssen Biotech Inc. to jointly develop and solely commercialize aprocitentan worldwide*
*** Market registration trials are being conducted in Japan*

Idorsia has the option to license vamorolone from ReveraGen Inc. and has granted to Santhera Holding Ltd. the option to sub-license vamorolone worldwide for all indications.

Human Resources

Idorsia created 93 new positions worldwide in 2018, bringing the total number of employees (permanent, post-doc, and apprentices) to 753 (2017: 660).

Annual Report

Full details on the progress made in 2018 are available in Idorsia's 2018 Annual Report, at www.idorsia.com/annual-report

Note to Shareholders

The Annual General Meeting (AGM) of Shareholders to approve the Annual Report of the year ending December 31, 2018 will be held on Friday May 3, 2019.

Registered shareholders with voting rights individually or jointly representing at least 5% of the share capital of the company, being entitled to add items to the agenda of the general meeting of shareholders, are invited to send in proposals, if any, to Idorsia Ltd, attention Corporate Secretary, Hegenheimermattweg 91, CH-4123 Allschwil, to arrive no later than March 14, 2019. Any proposal received after the deadline will be disregarded.

In order to attend and vote at the Annual General Meeting, shareholders must be registered in the company's shareholder register by April 24, 2019 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

- First Quarter 2019 Financial Results reporting on April 18, 2019
- Annual General Meeting of Shareholders on May 3, 2019
- Half-Year 2019 Financial Results reporting on July 23, 2019
- Nine-months 2019 Financial Results reporting on October 22, 2019

Notes to the editor

Letter to Shareholders (as published in Idorsia's 2018 Annual Report on February 7, 2019)

Advancing with speed and agility

Dear Shareholders

In 2018, Idorsia saw advances on all fronts:

- We advanced our pipeline, bringing four products into Phase 3 development.
- We completed demerger activities, with all core systems now running independently of Actelion.
- We appointed a leader to build our commercial organization, thus taking another step forward towards financial sustainability.
- We strengthened our cash position so as to be able to run our Phase 3 clinical trials and then take strategic decisions on how best to commercialize our assets.

Late-stage assets leap forward

Our late-stage pipeline advanced significantly in 2018, with four compounds moving into Phase 3 clinical development. All our late-stage trials are now initiated, with data expected to be reported in 2020 and 2021.

Lucerastat is an oral therapy offering a new treatment approach for patients with Fabry disease. Recruitment for the MODIFY trial started in May and the study is expected to report results in the first half of 2020.

ACT-541468 is a dual orexin receptor antagonist for the treatment of insomnia. It has the potential to deliver fast onset of sleep and a duration of action not exceeding a normal night, while preserving natural sleep architecture. Recruitment for the Phase 3 program started in June and we are on track to report results in the first half of 2020.

Aprocitentan – an orally active dual endothelin receptor antagonist – is being investigated in the PRECISION trial for patients whose hypertension is uncontrolled despite the use of at least three antihypertensive drugs. Patients were enrolled at the first site in June, and we are close to having all sites recruiting as patient screening progresses.

Clazosentan – a selective endothelin (ETA) receptor antagonist – is being developed for the prevention of clinical deterioration due to vasospasm-related delayed cerebral ischemia following aneurysmal subarachnoid hemorrhage. The global REACT study commenced enrollment at the beginning of February 2019.

Development of clazosentan is further advanced in Japan, where a Phase 3 program was initiated by Actelion in 2016; recruitment continues, and the studies are expected to be completed in the first half of 2020. To pave the way for bringing clazosentan to market, we have established Idorsia Japan. This has the additional advantage of allowing us to tailor our other development programs to include Japan at a very early stage.

Building the pipeline of the future
We are also making great progress with our early-stage compounds by accumulating information on clinical pharmacology in healthy volunteers or conducting Phase 2 profiling in patients.

Two Phase 2 studies with selatogrel, Idorsia's P2Y12 receptor antagonist, met their pharmacodynamic objectives of significantly inhibiting platelet aggregation and showed the desired profile for future development. We are preparing for the end of Phase 2 meetings with Health Authorities where we will discuss the Phase 3 study.

Another example is cenerimod, our selective $S1P_1$ receptor modulator, which is being investigated for the treatment of adults with systemic lupus erythematosus in a multiple-dose, efficacy and safety study. This is the first time $S1P_1$ receptor modulation has been studied in a large clinical trial for patients with lupus, potentially transforming the treatment landscape for this underserved patient population.

And there's much more to come, as Drug Discovery continues to generate the pipeline of the future.

Operational independence
On the first day after the demerger, Idorsia was operationally fully functional, meaning that our business, research and clinical development could continue without any interruption. However, though Idorsia was launched with a broad pipeline, an established team and with the necessary laboratories and workspaces, we were still reliant on certain services and systems provided by Actelion.

Over the course of 2018, we cut the umbilical cord and we are now fully independent: all of our core systems – from e-mail and data servers to support environments for research and clinical development – have been decoupled from the old infrastructure, and new, highly scalable state-of-the-art solutions have been implemented in all areas of the business.

Completing the value chain
In December 2018, Simon Jose joined the Idorsia Executive Committee as Chief Commercial Officer. He is now creating Idorsia's commercial infrastructure and executing on our strategic priority of being able to take our own research and innovation from the lab to patients. The establishment of a fully functional subsidiary in Japan constitutes a major first step in setting up a global commercial infrastructure.

Building a company with its own commercial infrastructure means that we are in control of the planning, development and implementation of commercial strategies for our assets. This in turn will give us financial independence and sustainability, thus maximizing the potential benefits for Idorsia.

Financing pipeline development
Rather than focusing all our efforts on a single drug, we have advanced all our late-stage assets into Phase 3 simultaneously. We believe that this approach gives us the best chance of achieving success, building the business, and funding future innovations. However, the level of financing required for a diversified drug pipeline can pose certain challenges for a young company. The CHF 505 million raised in July 2018 should give us the necessary leeway to assess the clinical data for our late-stage pipeline and then make the appropriate strategic decisions regarding commercialization.

Committed to creating long-term value
Building a company requires an investment of time, effort and money, as well as the confidence of shareholders. We will continue to make these investments to ensure that our innovative medicines can fulfill their potential for the benefit of patients around the world.

Sincerely,

Jean-Paul Clozel, Chief Executive Officer Jean-Pierre Garnier, Chairman of the Board

Issuance of new registered shares

In July 2018, Idorsia placed 11,912,000 new registered shares of CHF 0.05 par value each at CHF 25.62 per new share (the "Offer Shares") with gross proceeds of CHF 305 million. The Offer Shares, corresponding to approximately 10% of Idorsia's currently issued share capital, were issued from Idorsia's existing authorized share capital and ranked pari passu with the existing shares. The listing and the admission to trading of the Offer Shares according to the International Reporting Standard of the SIX Swiss Exchange was effective on July 13, 2018.

Issuance of senior unsecured convertible bonds

In July 2018, Idorsia placed CHF 200 million of senior unsecured convertible bonds (the "Bonds"), due 2024. The Bonds have a maturity of 6 years and are convertible into 5.9 million registered shares of Idorsia, sourced from existing listed conditional share capital, on or after August 27, 2018. The Bonds have a coupon of 0.75%, subject to Swiss withholding tax, and a conversion price of CHF 33.95, corresponding to a conversion premium of 32.5% above the book-building price of the Offer Shares. Holders of the Bonds who convert their Bonds will receive Idorsia shares with a par value of CHF 0.05 per Idorsia share. Idorsia will be entitled to redeem the Bonds at their principal amount (plus accrued interest) in accordance with the terms and conditions of the Bonds at any time (i) on or after August 7, 2022, if the price of an Idorsia share is equal to or exceeds 150% of the then prevailing conversion price over a certain period or (ii) if less than 15% of the aggregate principal amount of the Bonds remains outstanding. The Bonds, unless previously converted or repurchased and cancelled, will be redeemed at 100% of their principal amount.

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 750 highly qualified specialists dedicated to realizing our ambitious targets.

For further information, please contact

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