Media Release

06 February 2018

Idorsia announces financial results for 2017 – company off to a great start – 4 compounds to enter Phase 3 development

Allschwil, Switzerland – 06 February 2018 – Idorsia Ltd (SIX: IDIA) today announced its financial results as of December 31, 2017, which represent the business activities for the six and a half months since the demerger from Actelion on June 15, 2017.

Jean-Paul Clozel, CEO of Idorsia, commented: "We have made great progress in establishing Idorsia, while simultaneously advancing our pipeline without any loss of momentum. We have also entered into meaningful collaborations with industry leaders Janssen Biotech and Roche. Looking ahead, I am very excited by the prospect of advancing four compounds into Phase 3 development this year. With our substantial pipeline and these strong partnerships, we have every reason to be optimistic about our future."

Key figures

- US GAAP operating results 2017: YTD* loss of CHF 8 million; Q4 profit of CHF 71 million
- Non-GAAP** operating income 2017: YTD CHF 8 million; Q4 CHF 79million
- Non-GAAP operating expenses 2017: YTD CHF 150 million; Q4 CHF 79 million
- Guidance for 2018: Non-GAAP operating expenses around CHF 390 million

At the end of 2017, Idorsia's liquidity (including cash, cash equivalents, short- and long-term deposits) amounted to CHF 1,091 million.

	Period ending Dec 31, 2017		Fourth quarter 2017	
(in CHF millions, except EPS)	US GAAP	Non-GAAP	US GAAP	Non-GAAP
Revenues	158	158	158	158
Operating expenses	(166)	(150)	(87)	(79)
Operating income (loss)	(8)	8	71	79
Net income (loss)	(14)	5	68	77
Basic EPS	(0.13)	0.04	0.57	0.65
Basic number of shares (weighted average)	114.0	114.0	119.1	119.1
Diluted EPS	(0.13)	0.03	0.43	0.49
Diluted number of shares (weighted average)	114.0	139.5	157.9	157.9

***** *Year-to-date results correspond to the six and a half months operations since the demerger from Actelion.*

****** *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.*

Financial results

For the period ending December 31, 2017, US GAAP operating loss amounted to CHF 8 million and Non-GAAP operating income amounted to CHF 8 million. US GAAP operating loss is based on revenues of CHF 158 million, R&D expenses of CHF 123 million, G&A expenses CHF 27 million, depreciation and amortization CHF 10 million, and share-based compensation CHF 6 million.

The US GAAP net loss amounted to CHF 14 million resulting in a net loss per share of CHF 0.13.

André C. Muller, CFO of Idorsia, commented: "We have a diverse pipeline with unique assets that offer significant potential to patients and investors alike. We must invest in this pipeline now in order to make Idorsia a profitable company, in a sustainable manner, as quickly as possible. With this in mind, unforeseen events and potential milestone expenses excluded, we expect non-GAAP operating expenses for 2018 to be around 390 million Swiss francs, mainly depending on when each of the different Phase 3 programs commences."

Liquidity and indebtedness

(in CHF millions)	Dec 31, 2017	Sep 30, 2017	Jun 30, 2017
Liquidity			
Cash and cash equivalents	622	552	607
Short-term deposits	218	150	150
Long-term deposits	250	250	250
Total Liquidity	**1,091**	**952**	**1,007**
Indebtedness			
Convertible loan	365	363	361
Other financial debt	-	-	-
Total indebtedness	**365**	**363**	**361**

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.

Compound	Mechanism of Action	Target Indication	Status
Aprocitentan*	Dual endothelin receptor antagonist	Resistant hypertension management	Advancing to Phase 3
ACT-541468	Dual orexin receptor antagonist	Insomnia	Advancing to Phase 3
Clazosentan**	Endothelin receptor antagonist	Vasospasm associated with aneurysmal subarachnoid hemorrhage (aSAH)	Advancing to Phase 3
Lucerastat	Glucosylceramide synthase inhibitor	Fabry disease	Advancing to Phase 3
Cenerimod	$S1P_1$ receptor modulator	Systemic lupus erythematosus	Phase 2
Vamorolone***	Dissociative steroid	Duchenne muscular dystrophy	Phase 2
ACT-246475	P2Y12 receptor antagonist	Acute coronary syndrome (ACS)	Phase 2
ACT-774312	CRTH2 receptor antagonist	Asthma and allergy disorders	Phase 1
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 to jointly develop and solely commercialize aprocitentan worldwide*
*** In Japan, market registration trials are being conducted, with results expected in the second half of 2018*
**** Idorsia has exclusive option to worldwide rights to ReveraGen's Vamorolone.*

In 2017, several Phase 2 studies were concluded and the company engaged with regulatory authorities to further advance these compounds. In the course of 2018, Idorsia aims to move four of these projects into Phase 3 clinical development.

Lucerastat for Fabry disease

In an exploratory study in patients with Fabry disease, treatment with lucerastat in addition to ERT demonstrated a marked decrease in plasma levels of metabolic substrates associated with the disease. The study also demonstrated that lucerastat is well tolerated in patients with Fabry disease.

In the first half of 2018, Idorsia expects to initiate a pivotal Phase 3 study designed to assess the effects of lucerastat on neuropathic pain and gastrointestinal symptoms, as well as safety

and tolerability, in patients with Fabry disease. The study is expected to enroll around 100 patients and to last approximately 20 months.

Lucerastat for Fabry disease has received Orphan Drug designation in the US and in the EU and at the beginning of 2018, the European Medicines Agency (EMA) agreed with Idorsia's paediatric investigation plan for lucerastat for the treatment of pediatric patients with Fabry disease. Idorsia has already initiated activities according to the agreed plan.

ACT-541468 for insomnia

The safety and efficacy of ACT-541468 in adult and elderly patients with insomnia was evaluated in a comprehensive Phase 2 program, comprising two studies. Both studies showed the desired effect on sleep maintenance and onset, with a significant dose-response relationship; treatment was generally well tolerated.

In the first half of 2018, following feedback from health authorities, Idorsia expects to initiate a pivotal Phase 3 registration program. The program consists of three studies designed to evaluate time to sleep onset, sleep maintenance, and next day performance, as well as providing long-term safety information, in patients with insomnia.

Aprocitentan for resistant hypertension management

In a Phase 2 study – completed in May 2017 – the efficacy, safety and tolerability of aprocitentan was evaluated in patients with essential hypertension in order to identify the optimal dose for further studies.

Based on the positive results from the dose-finding study and following feedback from health authorities, Idorsia is currently finalizing the design of a Phase 3 study. This will be specifically designed to evaluate the initial and long-term effects of aprocitentan on systolic and diastolic blood pressure in patients requiring resistant hypertension management (RHM). The study is expected to start in the first half of 2018. If successful, it will provide the basis for registration of the product.

On December 1, 2017, Janssen Biotech, Inc. (one of the Janssen Pharmaceutical Companies of Johnson & Johnson) exercised its option to enter into a collaboration agreement with Idorsia to jointly develop and commercialize aprocitentan and any of its derivative compounds or products. Idorsia received a one-time milestone payment of USD 230 million. Both parties have joint development rights over aprocitentan. Idorsia will oversee Phase 3 development and regulatory submission for the first indication. The costs will be shared equally between the two partners. Janssen will oversee Phase 3 development and submission for any additional indications.

Clazosentan for cerebral vasospasm associated with aneurysmal subarachnoid hemorrhage (aSAH)

Several studies have built our understanding of the effects of clazosentan on cerebral vasospasm, indicating that it has the potential to prevent ischemic complications of cerebral vasoconstriction and to decrease the need for invasive endoarterial intervention.

In Japan, two registration studies evaluating the safety and efficacy of clazosentan in reducing vasospasm-related morbidity and mortality events after aneurysm-securing procedures are being conducted. Results are expected in the second half of 2018.

Later in 2018, Idorsia expects to initiate a Phase 3 study evaluating the safety and efficacy of clazosentan in an aSAH population enriched for the risk of cerebral vasospasm.

Cenerimod for systemic lupus erythematosus

In a Phase 2 study in adult patients with SLE, cenerimod induced a dose-dependent reduction in lymphocyte count and was well tolerated at all dose levels.

In December 2017, the US FDA designated the investigation of cenerimod for the treatment of systemic lupus erythematosus as a Fast Track development program. The Fast Track designation is intended to promote communication and collaboration between the FDA and the company for drugs that treat serious conditions and fill an unmet medical need.

Idorsia is currently discussing the development program with health authorities to advance cenerimod in this underserved disease as quickly as possible.

Human Resources

At the end of 2017, Idorsia employed 660 employees (permanent, post-doc, and apprentices) worldwide.

Annual Report

Full details on the progress made in 2017 are available in Idorsia's 2017 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, 2017 will be held on Tuesday April 24, 2018.

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 5, 2018. Any proposal received after the deadline will be disregarded.

In order to attend and vote at the Annual General Meeting of Shareholders, shareholders must be registered in the company's shareholder register by April 13, 2018 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

- 3 Months 2018 Financial Results reporting on 19 April 2018
- Half-Year 2018 Financial Results reporting on 24 July 2018
- 9 Months 2018 Financial Results reporting on 23 October 2018

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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 intend to develop Idorsia into Europe's leading biopharmaceutical company, with a strong scientific core.

Headquartered in Switzerland - a European biotech hub - 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 SIX Swiss Exchange (ticker symbol: IDIA) in June 2017 and has over 650 highly qualified specialists dedicated to realizing our ambitious targets.

For further information, please contact:

Andrew C. Weiss
Senior Vice President, Head of Investor Relations & Corporate Communications
Idorsia Pharmaceuticals Ltd, Hegenheimermattweg 91, CH-4123 Allschwil
+41 58 844 10 10
www.idorsia.com

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.