



**Full Year 2018**

**Financial and Corporate  
Update**

# Disclaimer

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# Well Diversified Mid-to-Late Stage Metabolic Pipeline for Large Market Opportunities

Global partnerships secured for late stage clinical program in type 2 diabetes

- **Imeglimin:** First in class oral drug candidate targeting mitochondrial dysfunction
  - Partnered with Sumitomo Dainippon Pharma and Roivant Sciences
  - Phase 3 data results for Japan beginning early Q2 2019; Phase 3 initiation in US 2019
  - Total deal value >\$900M plus royalties; partners funding Phase 3 and commercialization

Two clinical stage NASH programs targeting underlying root cause of disease which can be developed as monotherapy and/or as combination therapy

- **PXL770:** Direct AMPK activator for NASH
  - Pathway targeting steatosis, inflammation, and fibrosis
  - Phase 2a program initiation Q1'19
- **PXL065:** MPC inhibitor for NASH
  - Deuterium-stabilized R-pioglitazone
  - Pioglitazone (racemate) demonstrated resolution of NASH without worsening of fibrosis
  - Phase 1 ongoing; Pivotal Phase 2 program initiation Q4 19/Q1 20
- Preclinical studies underway for additional metabolic and rare diseases
- Euronext listed (POXEL); strong cash position
  - EUR 66.7 million (USD 76.4 million) cash & equiv. 12/31/18; runway into 2021

# Metabolic Pipeline

## Well-diversified Pipeline with Mid-to-late-stage Programs

	Indication	MOA	Preclinical	Phase 1	Phase 2	Phase 3	Partner/ Rights	Next Steps	
<b>Imeglimin Japan/ Asia*</b>	Type 2 Diabetes	Mitochondrial Bioenergetics					Ph 3		<ul style="list-style-type: none"> <li>Phase 3 TIMES completion</li> <li>Target JNDA submission 2020</li> </ul>
<b>Imeglimin US/ EU/ Other**</b>	Type 2 Diabetes	Mitochondrial Bioenergetics					Ph 3		<ul style="list-style-type: none"> <li>Manufacturing drug for Phase 3</li> <li>Study in T2D patients w/ CKD</li> </ul>
<b>PXL770</b>	NASH/ metabolic diseases	Direct AMPK activator					Ph 2		<ul style="list-style-type: none"> <li>Initiate Phase 2a program in NASH</li> </ul>
<b>PXL007 (EYP001)</b>	Hepatitis B NASH	FXR agonist					Ph 2		<ul style="list-style-type: none"> <li>Complete Phase 1 program by Enyo Pharma</li> </ul>
<b>PXL065 (formerly DRX-065)</b>	NASH	MPC Inhibitor					Ph 2		<ul style="list-style-type: none"> <li>Complete Phase 1, tox, CMC</li> <li>Initiate Pivotal Phase 2 study</li> </ul>
<b>Poxel/ DeuteRx programs</b>	Metabolic (AMN/ALD, NASH, etc.)	Direct AMPK activator/ MPC Inhibitor					Ph 1		<ul style="list-style-type: none"> <li>Complete preclinical studies</li> </ul>

*Open arrow designates expected development status in 2019*

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\*including: China, South Korea, Taiwan, Indonesia, Vietnam, Thailand, Malaysia, the Philippines, Singapore, Myanmar, Cambodia, and Laos.

\*\*countries not covered in the Sumitomo Dainippon Pharma agreement



A close-up photograph of a microscope with a person's hands wearing blue nitrile gloves. The hands are positioned around the microscope's stage and objective lenses. The background is blurred, showing a laboratory setting.

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**Financial Update**

## STATEMENTS OF COMPREHENSIVE INCOME (LOSS) – IFRS – in KEUR

	At December, 31	
	2018	2017
<b>Revenue</b>	<b>74 605</b>	<b>5 290</b>
Research and development		
Research and development Expenses	-58 092	-24 096
Tax Credit	3 552	3 122
General and administrative	-7 527	-6 219
<b>Operating expenses</b>	<b>-62 067</b>	<b>-27 192</b>
<b>Operating Income</b>	<b>12 538</b>	<b>-21 902</b>
<b>Financial income (loss)</b>	<b>1 064</b>	<b>-396</b>
<b>Net income (loss) before tax</b>	<b>13 602</b>	<b>-22 298</b>
Income tax	-77	
<b>Net income loss</b>	<b>13 525</b>	<b>-22 298</b>

# Statements of Financial Position (IFRS) – in KEUR

(amounts in k€)	At December 31, 2018	At December 31, 2017
Intangible assets	16 577	0
Property, plant and equipment	296	143
Other financial assets	372	356
Deferred tax assets		
<b>Total non-current assets</b>	<b>17 246</b>	<b>500</b>
Trade receivables	14 262	4 902
Other receivables	7 271	7 187
Cash and cash equivalents	66 737	54 163
<b>Total current assets</b>	<b>88 270</b>	<b>66 253</b>
<b>Total assets</b>	<b>105 516</b>	<b>66 752</b>

(amounts in k€)	At December 31, 2018	At December 31, 2017
<b>Total shareholder's equity</b>	<b>55 782</b>	<b>19 327</b>
<b>Non-current liabilities</b>		
Employee benefit obligations	279	230
Financial liabilities	359	555
<b>Total non-current liabilities</b>	<b>638</b>	<b>785</b>
Financial liabilities	226	936
Provisions	18	84
Trade payables	20 742	9 008
Other current liabilities	28 110	36 613
<b>Total current liabilities</b>	<b>49 096</b>	<b>46 640</b>
<b>Total liabilities and shareholders' equity</b>	<b>105 516</b>	<b>66 752</b>



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**Corporate Update**



# 2018 Major Accomplishments

## Imeglimin

- ① Strategic partnership with Roivant Sciences
  - Phase 3-related activities with Metavant, Roivant's subsidiary
- ② Significant progress in Japan
  - Phase 3 TIMES program (3 pivotal trials) fully enrolled in Japan

## PXL770

- ③ Data presented at several scientific Congresses
- ③ Phase 1b program successfully completed
- ④ Preparation for Phase 2a program

## PXL065

- ⑤ Strategic agreement with DeuteRx for clinical stage NASH drug candidate, and other programs
- ⑤ Advanced PXL065 to Part 2 of Phase 1a study

## Corporate

- ⑥ Added to the depth of management team and established a subsidiary in Japan and the US
  - Appointed Takashi Kaneko, MD, PhD as Senior Vice President Medical and President of Poxel Japan K.K.

# Full Year Half 2018 Highlights: Roivant Partnership for US/Europe/and other Countries Worldwide\*

- Roivant is committed to developing innovative therapies for major disease areas, including type 2 diabetes
- Imeglimin will be a cornerstone program in Metavant
- Total deal value is \$625M (~€507M)
  - \$35M upfront payment
  - \$15M (~€12M) investment at €8.5 per share
  - Up to \$600M (~€486M) in future potential development and regulatory milestone payments and sales-based payments
  - Escalating double-digit royalties on net sales
- Roivant is responsible for Imeglimin's development and commercialization in the U.S., Europe, and other countries\*
  - Poxel contributing \$25M (~€20M) to development program over a 2-year period
- Poxel and Roivant to decide on a potential co-promotion prior to commercialization
- Roivant to develop Imeglimin (RVT-1501) initially to treat patients with type 2 diabetes with chronic kidney disease (CKD) stages 3b/4<sup>1</sup>
  - Opportunity to study Imeglimin in broader T2D population

# Full Year 2018 Highlights: Roivant Development Focus for Imeglimin

- Diabetes is the most common cause of chronic kidney disease
- Patients with type 2 diabetes and CKD stages 3b/4<sup>1</sup>
  - Approximately 2.4 million adults in the US<sup>2</sup>
  - Challenging glucose management
- Underserved population
  - Many approved therapies require dose reduction or are not recommended in the presence of kidney disease
  - Insulin and insulin secretagogues are the most commonly used therapies at suboptimal doses to prevent risk of hypoglycemia
  - Need for a new treatment at optimal dose, providing a strong efficacy and safety profile with no hypoglycemia risk
- Imeglimin Phase 2 data (Japan & US) showed similar safety & efficacy in patients with impaired renal function compared to patients with normal renal function
- Phase 3 program-related work conducted in 2018
  - Study ongoing in patients with T2D and moderate-to-severe chronic kidney disease
  - Manufacturing of drug product for use in Phase 3 program
- Goal is to initiate Phase 3 Program in 2019

1 CKD stage 3b= eGFR 30-44 ml/min/1.73 m<sup>2</sup> inclusive; CKD stage 4 = 15-29 ml/min/1.73m<sup>2</sup> inclusive

2 Centers for Disease Control and Prevention (CDC). NCHS. NHANES. Laboratory Data, 2015-2016. Hyattsville, MD: U.S. Department of Health and Human Services, Centers for Disease Control and Prevention, 2017.

## 2 Full Year 2018 Highlights: Imeglimin Phase 3 progress in Japan



- Imeglimin Phase 3 TIMES program significantly progressed in 2018
  - Enrollment of >1,100 patients in Phase 3 program completed August 2018
    - TIMES 1: multicenter, double-blind, placebo-controlled, randomized, monotherapy study in >200 Japanese patients: **Topline results expected early Q2 19**
    - TIMES 2: 52-week, open-label, parallel-group study in >700 Japanese patients assessing long-term safety and efficacy. Administrated as mono- or combo therapy w/hypoglycemic agents: DPP4, SGLT2, biguanide, sulphonylurea and GLP1: **Topline results expected Q4 19**
    - TIMES 3: 16-week double-blind, placebo-controlled, randomized study with 36 week open-label extension evaluating efficacy and safety with insulin in >200 Japanese patients and inadequate glycemic control on insulin therapy: **Topline results expected for 16-week portion expected mid-year; full results Q4 19**
- New data presented at American Diabetes Association 78<sup>th</sup> Scientific Sessions
  - Imeglimin observed to protect and preserve human beta-cells from cell death from fructose- and glucose-induced toxicity by inhibiting mPTP
  - Data highlights potential to delay type 2 diabetes disease onset and progression through preservation of beta-cell mass

# Full Year 2018 Highlights: PXL770 for NASH

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- Preclinical PoC data presented at Global NASH Congress 2018
  - PXL770 observed to significantly reduce liver steatosis and NAS score following eight weeks of treatment vs control and significantly reduce expression of a panel of key genes associated with fibrosis
- Data presented at AASLD Congress 2018
  - Showed beneficial effect on both the adipose tissue and liver through direct activation of AMPK in a DIO-NASH model
- Clinical and preclinical data presented at AMPK Congress in 2018
  - PXL770 observed to have a favorable pharmacokinetic, tolerability and safety profile in Phase 1 and a favorable cardiac safety profile in animal models
- Phase 1b multiple ascending dose (MAD) trial was completed
  - PXL770 observed to have favorable safety and PK profile (n=48)
- Preparations were underway in 2018 for Phase 2a program

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- The Phase 2a program to include two separate studies
- Phase 2a efficacy and safety study; expected to initiate Q1 19 with results anticipated 1H 20
- PK/PD study expected to initiate 2Q 19 with results anticipated 2H 19

# Full Year 2018 Highlights : Strategic Agreement for NASH drug candidate, and other programs



- 5 • Poxel acquired exclusive, worldwide ownership to PXL065 (deuterium-stabilized R-pioglitazone) and additional programs from DeuteRx
  - Phase 1 program for the treatment of NASH
- Based upon preclinical and Phase 1 data, PXL065 anticipated to show a better therapeutic profile than pioglitazone
  - Potential for similar efficacy with reduction of side effects, such as those associated with PPAR- $\gamma$  activation
- Acquired additional programs, including deuterated drug candidates for metabolic, specialty and rare diseases
  - Exploring other opportunities to advance from the DeuteRx metabolic portfolio, including those for rare diseases
- PXL065 data presented at AASLD suggest potential for similar efficacy with a reduced side effect profile from pioglitazone for NASH
- Initiated Part 2 of PXL065 Phase 1a study
  - Study will assess safety and tolerability with secondary objective to assess dose proportionality
- 5 • Preparation underway for Phase 1b multiple ascending dose (MAD) study expected to initiate in Q2 19

# Significant Upcoming Milestones for 2019/2020

- Imeglimin
  - Phase 3 TIMES 1 data readout (early Q2 19)
  - Phase 3 TIMES 3 16-week, double-blind, placebo-controlled part (Mid-19)
  - Phase 3 TIMES 2 and full results from TIMES 3 (Q4 19)
  - Imeglimin manuscripts published related to efficacy, safety and PK (2019)
  - Roivant Phase 3 initiation goal (2019)
  - NDA submission in Japan (2020)
  - Imeglimin target launch in Japan (2021)
- PXL770
  - Phase 2a efficacy and safety study initiation (Q1 19)
  - PK/PD study initiation (Q2 19)
  - PK/PD data results (2H 2019)
  - Phase 2a data results (1H 20)
- PXL065
  - Initiate Phase 1b multiple ascending dose study (Q2 19)
  - Completion of Phase 1 program (Mid-year to third quarter 19)
  - Pivotal Phase 2 initiation in NASH (Q4 19 / Q1 20)
  - Pivotal Phase 2 readout (2022)
- 15 Additional preclinical data on other metabolic and rare diseases (2019)



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