

Sijmen de Vries Chief Executive Officer

> IEX Beleggersdag Amsterdam 01 June 2018



#### Safe Harbour Statement

The information contained in this document and communicated verbally to you (together the "Presentation") is being supplied to you solely for your information and may not be copied, reproduced or further distributed to any person or published, in whole or in part, for any purpose.

The Presentation does not form any part of an offer of, or invitation to apply for, securities in Pharming Group N.V. (the "Company").

The Presentation speaks as of the date shown on the front cover. The Company assumes no obligation to notify or inform the recipient of any developments or changes occurring after the date of this document that might render the contents of the Presentation untrue or inaccurate in whole or in part. In addition, no representation or warranty, express or implied, is given as to the accuracy of the information or opinions contained in the Presentation and no liability is accepted for any use of any such information or opinions given by the Company or by any of its directors, members, officers, employees, agents or advisers.

The Presentation contains forward-looking statements, including statements about our beliefs and expectations. These statements are based on our current plans, estimates and projections, as well as our expectations of external conditions and events. Forward-looking statements involve inherent risks and uncertainties and speak only as of the date they are made. The Company undertakes no duty to update these and will not necessarily update any of them in light of new information or future events, except to the extent required by applicable law.

The Company's securities have not been and will not be registered under the U.S. Securities Act of 1933, as amended (the "Securities Act"), and may not be offered or sold in the United States absent registration under the Securities Act or an available exemption from, or transaction not subject to, the registration requirements of the Securities Act.





#### Overview

- Euronext: PHARM market capitalization: ~€750 million
- Headquarters in NL, R&D in France, EU and US commercial operations with approximately 145 employees in total
- 1st product approved and marketed: RUCONEST®
  - Recombinant human C1-esterase inhibitor (enzyme replacement therapy)
  - For acute angioedema attacks in patients with hereditary angioedema (HAE)
  - Marketed in USA, EU, Korea and Israel with additional territories coming
- Platform technology to develop and produce (complex) recombinant human proteins

We develop and commercialize human therapeutic proteins for innovative therapies meeting important unmet patient needs



## Corporate Highlights

### RUCONEST® Commercialisation

- Re-acquisition of US commercialization rights from Valeant in December 2016
- Q1 2018 revenues: €29.5 million (Q1 2017: €15.5 million)
- Temporary supply issues during Q4 2017 at a competitor now resolved

# RUCONEST® Franchise Development

- Prophylaxis of HAE with published data showing efficacy as good as any
- sBLA accepted for review by FDA: Action date 21 September 2018
- Additional large (non-HAE-related) indications being assessed for RUCONEST®

# Maturing pipeline beyond RUCONEST®

- Program for α- glucosidase (ERT for Pompe's disease) filing for IND end of this year
- Use same transgenic founder technology to target \$1 billion+ market where all existing products have box warnings

#### Solid Financial Base

- Financed with a \$100 million 4yr facility with OrbiMed Advisors in July 2017
- Q1 2018 operating profit: €8.2 million (Q1 2017: €3.9 million)
- Q1 2018 net profit: €3.3 million (Q1 2017: net loss €5.7 million)
- Positive cashflows: Cash balance at Q1 2018: €60.0 million (Q1 2017 €27.6 million)

# US HAE Market: Rapid Growth, Significant Potential, Very Competitive

Total Market in \$millions	Competitive	
	The US HAE market is expected to continue to grow 20%+ p.a. until 2020***	
2,000		
	HAE disease awareness in the US continues to improve with more patients seeking relief for moderate symptoms***	
1,500		Shire
	Annual sales for Prophylaxis of HAE attacks >US\$700M*	CSL
1,000		
		Shire
500	Annual sales Acute Treatment of HAE attacks >US\$850M * **	CSL- Behring
		Pharming

- \* 2016 results/ SEC filings SHPG, Pharming
- \*\* Excludes plasma derived C1- esterase inhibitor sales / not disclosed by CSL Behring
- \*\*\* Leerink Swann, competitor interviews, 13 September 2012



Opportunity for Rationalisation of Treatment

RUCONEST as first and only recombinant (non-plasma) ERT is based on Pharming's very scalable platform

Several Investigator Sponsored Studies in additional indications are underway and initial results from first of these is expected in Q3 2018

Company-driven clinical development plan for an undisclosed additional indication to be initiated and first patients are to be treated within 2018

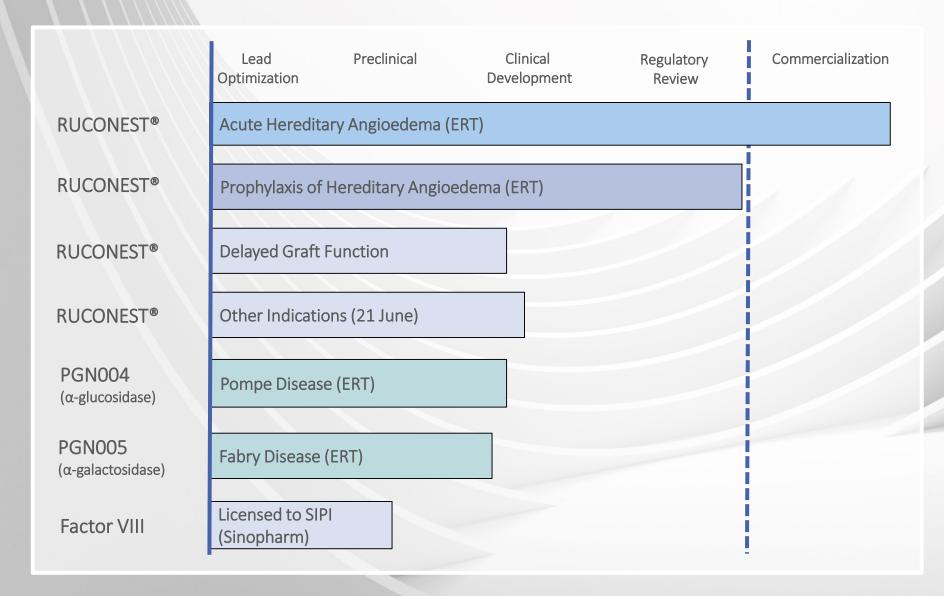
Very difficult for plasma-derived C1 esterase inhibitors to tackle these indications because of the limitations on available donations

Capital Markets Briefing on the new indications and development progress on 21 June 2018 In New York City / live webcast





# Building a multi-product franchise and pipeline



#### 12 Months Outlook

- Continued growth in sales of RUCONEST® driven by the US and EU operations
- Continuation of positive trend in operating results
- Continuation of positive Net Earnings during the year
- Continued investment in the expansion of production of RUCONEST
- Research and (Clinical) Development investments:
  - RUCONEST® in HAE (SC/IC/IM) with low volume vial to start by end 2018
  - Additional indications for RUCONEST® to start by end of 2018
  - New pipeline: Clinical development Pompe disease early 2019
- Increasing marketing activity, such as opening new countries for RUCONEST®
- Continue to support all our marketing partners to maximize the sales and distribution potential of RUCONEST® for patients in all territories

Increasing sales and continued positive results

www.pharming.com

Tickers:

ENXTAM: PHARM

Bloomberg: PHAR.AS

