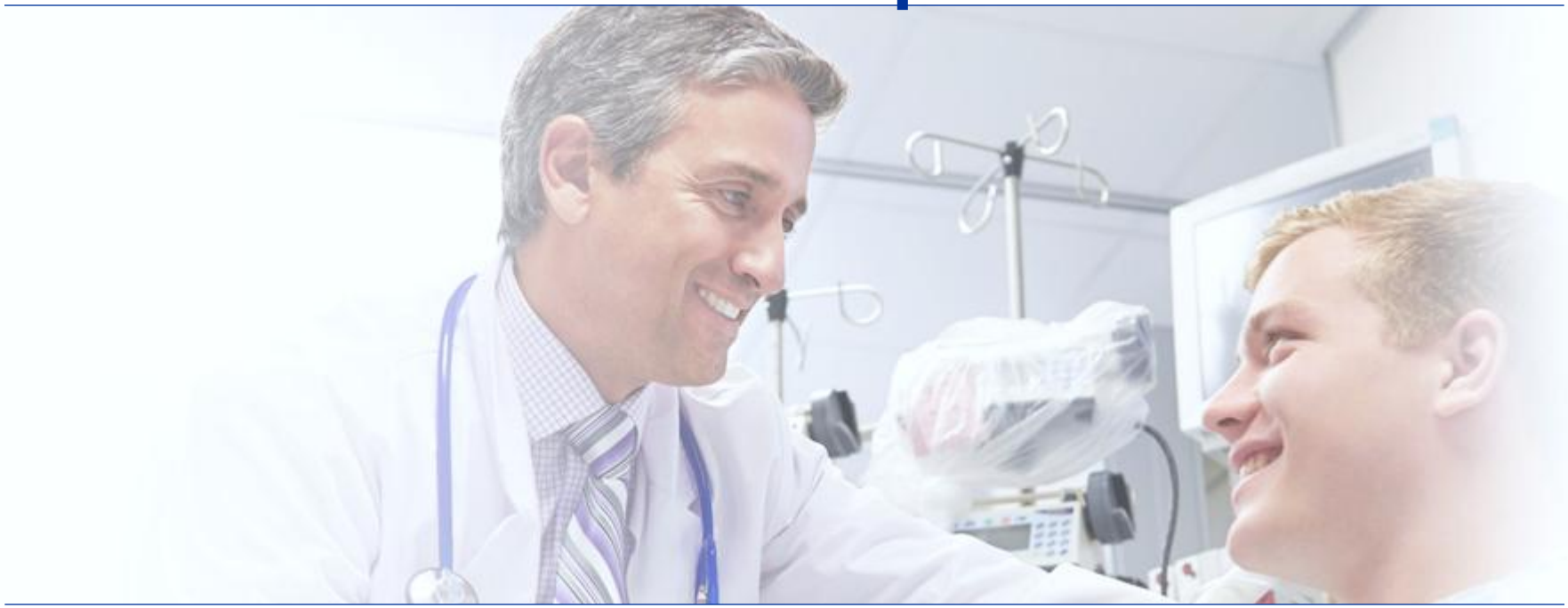
PAION H1 2017 Financial Results and Business Update



Dr. Wolfgang Söhngen, CEO | Abdelghani Omari, CFO

Conference Call | 9 August 2017

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Agenda

1. **Corporate overview**
2. Highlights YTD 2017
3. Financials
4. Remimazolam Update
5. Business Outlook

Corporate overview

PAION AG is a specialty pharma company with a focus on anesthesia products

Remimazolam, PAION's lead drug candidate, is in Phase III development

Seven regional partnerships for remimazolam in the U.S., China, South Korea, Canada, Russia (CIS), Turkey and MENA region

Headcount of 31, HQ in Aachen, Germany: PAION AG with a subsidiary in Cambridge (UK): PAION UK Ltd

~ EUR 27.1 million cash and cash equivalents (end of H1 2017)

Market capitalization: EUR ~ 180 m

Supervisory Board

Dr. Jörg Spiekerkötter (Chairman)
Background: Former CFO, Schering AG, Organon

Dr. Karin Dorrepaal
Background: Former Schering AG Board Member

John Dawson
Background: CEO Oxford BioMedica

Dr. Dr. Irina Antonijevic
Background: CMO, vasopharm GmbH

Dr. Chris Tanner
Background: Executive Board Cosmo Pharmaceuticals

Management

Dr. Wolfgang Söhngen
Title: CEO, Founder

Abdelghani Omari
Title: CFO

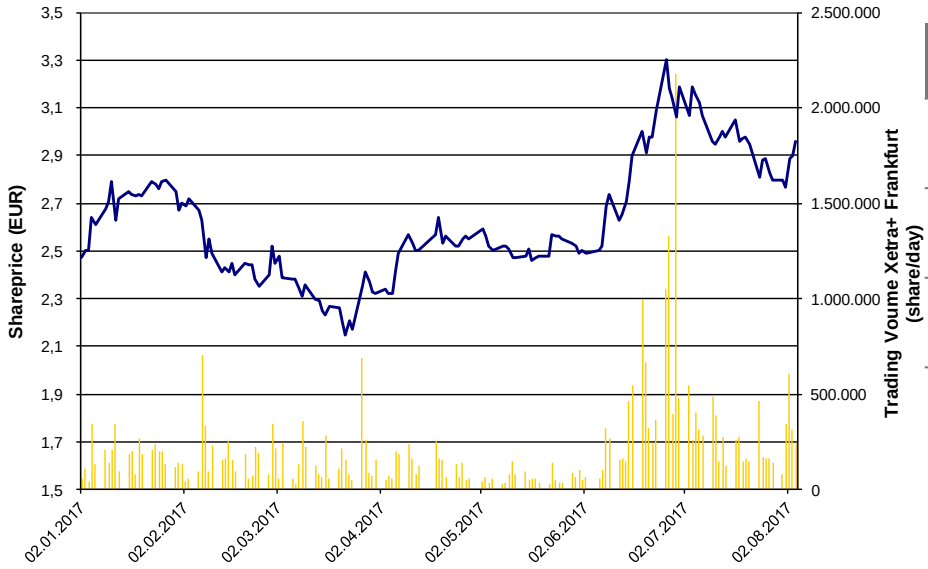
Stock Overview

Listed on Frankfurt Stock Exchange, Prime Standard
(FSE: PA8)

Market capitalization:
EUR ~ 180 m

High liquidity
(3-month period Xetra):
12 million shares traded

Stock Performance 2017 (price and volume in EUR)



Market Data (EUR in million except per share data)

Capitalization	
Current Share Price	€ 3.03
FD Shares Outstanding	61.1
Market Cap	€ 180
Mean target price of 3 analyst reports (Edison, Oddo Seydler, First Berlin)	€ 4.01

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Highlights – Year-to-date 2017 (1)

Important achievements driving our business forward

Positive results from two clinical trials with remimazolam

- U.S. Phase III trial in procedural sedation undergoing bronchoscopy
  - Primary efficacy endpoint successfully achieved
  - Excellent safety profile of remimazolam confirmed
- U.S. clinical safety trial in high-risk patients undergoing colonoscopy
  - Remimazolam administration appeared safe
  - Efficacy and efficiency gains comparable to confirmatory U.S. Phase III pivotal trials

Two financings successfully completed

- Total of ~EUR 13 million raised
- Financial position further improved

Supervisory Board changes resolved by the AGM reflect advancing development of company

- Dr. Dr. Irina Antonijevic – >16 years of drug development experience in various pharma organizations
- Dr. Chris Tanner – Cosmo Pharmaceuticals Board member, previously CFO

Highlights – Year-to-date 2017 (2)

Important achievements driving our business forward

Remimazolam partner Pharmascience had pre-NDS (New Drug Submission) meeting with Health Canada

- Non-clinical and clinical data package, including the human abuse liability data available at the time, were regarded as adequate for filing

PAION remimazolam licensing partner R-Pharm has started a Phase III trial in general anesthesia

- Multicentre, single-blind randomized comparative clinical trial of efficacy and safety of remimazolam and propofol in 150 surgery patients undergoing general anesthesia

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Two successful capital increases completed in 2017

EUR 5 million raised through capital increase with subscription rights

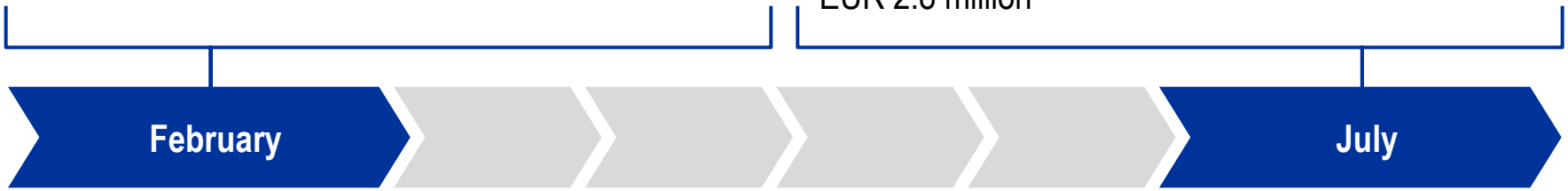
U.S. institutional investor agreed to backstop offering by purchasing all new shares that were not subscribed

Subscription rate was 96.31%

EUR 8 million raised trough private placement

U.S. institutional investor invested EUR 5.2 million; potential further subscription of up to 2.8 million new shares until 30 April 2018

Second largest shareholder TIAA-Cref invested EUR 2.8 million



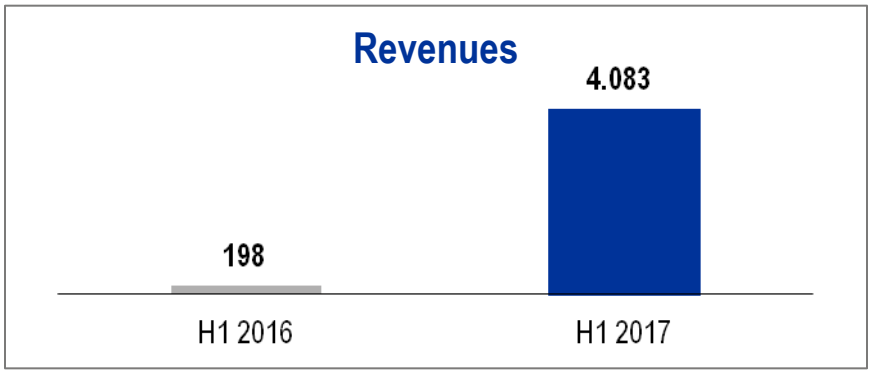
Total gross proceeds from financings: ~EUR 13 million

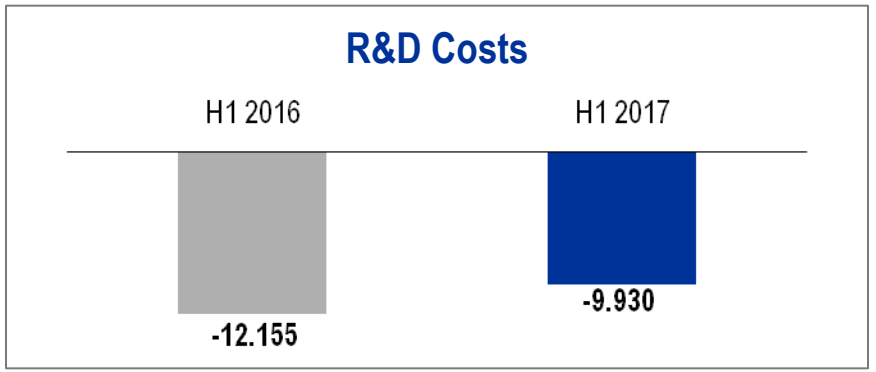
Strong financial position

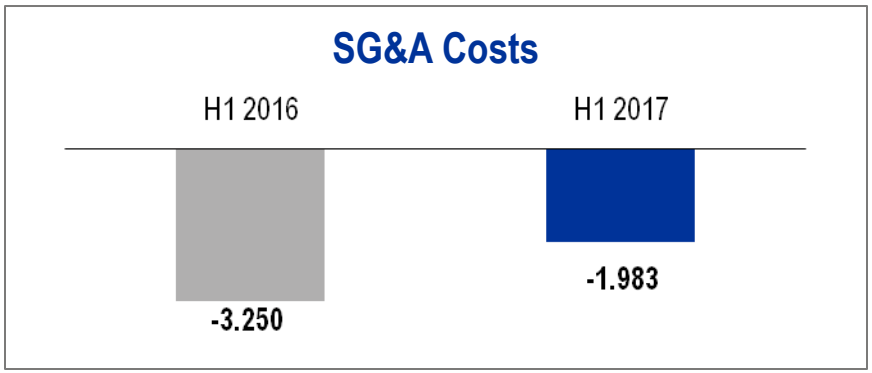
Total share capital of EUR 61.08 million after private placement in July 2017

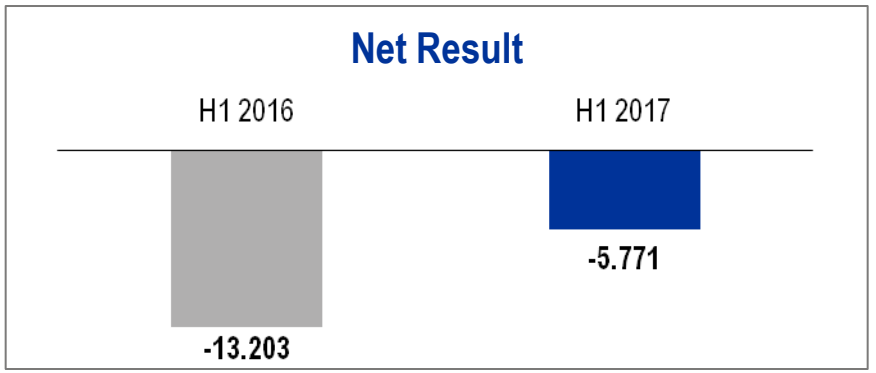
Consolidated statement of comprehensive income

In accordance with IFRS (all figures in EUR k)









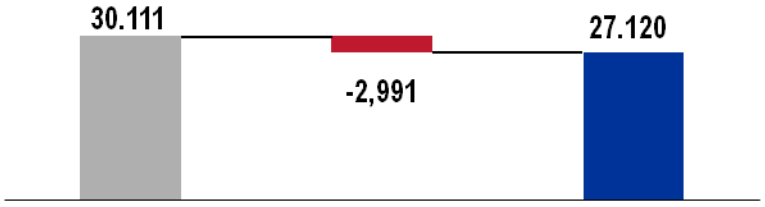
- Revenues mainly relate to upfront payment from Cosmo received in 2016
- R&D costs mainly relate to U.S. clinical development program; decrease primarily due to lower expenses for Phase III studies

Balance sheet and employees

In accordance with IFRS (all figures in EUR k if not otherwise noted)

Total assets

Cash and cash equivalents



Equity

Employees in the Group

→ Equity ratio as of 30 June 2017 was 73.4%

Financial Outlook 2017 – Guidance confirmed

		Actual FY 2016 EUR million	Plan FY 2017 EUR million	Comments
Revenues		€ 4.3 m	**~ € 5.8 m**	Revenues mainly relate to remainder of upfront payment received from Cosmo in 2016
Costs	R&D	€ 23.4 m	**~ € 18 m – 20 m**	Significant R&D expenses due to continuation of U.S. clinical development program of remimazolam, but lower than prior year
	SG&A	€ 5.1 m	**~ € 4 m**	SG&A costs will decrease compared to prior year
Tax credits		€ 5.0 m	**~ € 4 m**	Tax credits on portion of R&D expenses from UK tax authorities
Net result		€ -20.1 m	**~ € -12 m – -14 m**	Net loss will decrease

Liquidity

→ Sufficient cash to complete U.S. clinical development program in procedural sedation under current planning

→ Funding of approximately € 25 m for EU Phase III development until filing

→ Cash reach into second half of 2019

- Including gross proceeds of € 8 m collected from capital increase after balance sheet date
- Including expected UK tax credits on parts of research and development expenses
- Without consideration of potential incoming milestone payments
- Without consideration of potential costs for the targeted continuation of the EU Phase III development program

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Remimazolam – Positive results in three U.S. Phase III studies

- First-in-class ultra-short-acting intravenous benzodiazepine sedative/anesthetic
- Lead indication – Procedural sedation in the U.S.
  - Estimated market opportunity >$500 m globally for this indication alone
- IP:
  - Besylate salt – Protection until at least 2031 in the U.S.
  - Growing IP portfolio to secure attractive period of market exclusivity in major markets
- Extensive safety and efficacy database → over 1,500 volunteers/patients to date
- Results to date indicate:
  - Solid efficacy and safety in studied populations
  - Rapid onset and offset of action
  - Appropriate depth of sedation
  - Favorable hemodynamic stability

Remimazolam – Potential to address important issues

Need for improvement in the sedation/anesthesia space

- Improved overall safety
- Improved patient satisfaction and compliance
- Predictability of overall procedure time
- Improved patient throughput
- Reduced resources for supervision
- Avoidance of hypotension
- Amnesia for procedure with quick regaining of cognitive function
- "Back to normal" achieved quicker than with midazolam → improved compliance to follow-up screening
- Availability of a reversal agent
- Improved hemodynamic stability
- Less requirement for vasopressors
- Better suited for aging patient populations

Phase III bronchoscopy study overview procedural results

- Remimazolam outperformed the placebo arm and the open-label midazolam arm in the primary and all important secondary endpoints

	Remimazolam	Placebo	Midazolam (Open Label)
Procedural Success	82.5%	3.4%	34.8%
Use of Rescue Sedation	16.2%	96.6%	56.6%
Time from First Dose to Start of Procedure	5.0 min	17.0 min	16.0 min
End of Procedure to Fully Alert	6.0 min	14.0 min	12.0 min
Time to “back to normal”	404.0 min	935.0 min	478.5 min

Demographics

	Remimazolam Phase III bronchoscopy	Remimazolam Phase III colonoscopy	Remimazolam ASA III/IV colonoscopy
Mean age [years]	**62.3**	**54.9**	**62.5**
Age group >= 65 years [%]	**48.5**	**13.8**	**40.3**
Gender male vs. female [%]	45.9 vs. 54.1	47.6 vs. 52.4	55.8 vs. 44.2
Mean height [cm]	168.6	169.6	170.2
Mean weight [kg]	80.8	83.1	90.4
Mean BMI [kg / m^2]	28.3	29.0	30.8
ASA III [%]	**37.6**	**6.6**	**51.9**

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“The amazing remimazolam triplets”

Three U.S. trials with positive results

Phase III trial in procedural sedation in patients undergoing colonoscopy (n=461)

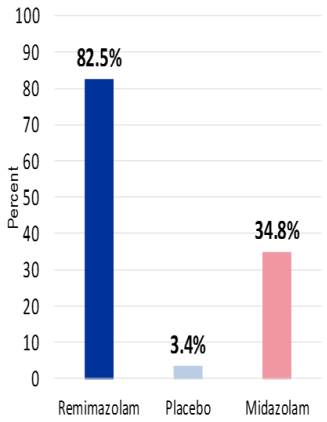
Phase III trial in procedural sedation in patients undergoing bronchoscopy (n=446)

Safety study in ASA III/IV (high-risk) patients undergoing colonoscopy (n=79)

Comparable outcomes in different patient populations

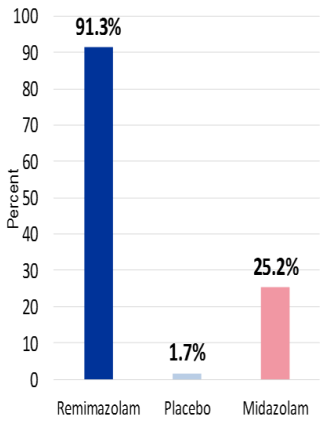
Phase III bronchoscopy (ASA I-III)

Success of procedure



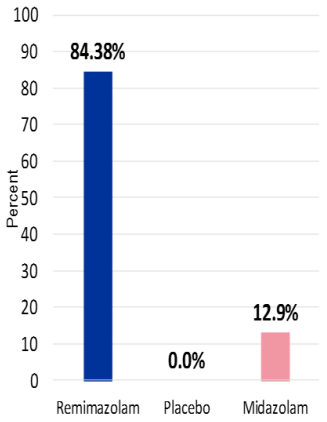
Phase III colonoscopy (mostly ASA I-II)

Success of procedure



ASA III/IV patients

Success of procedure



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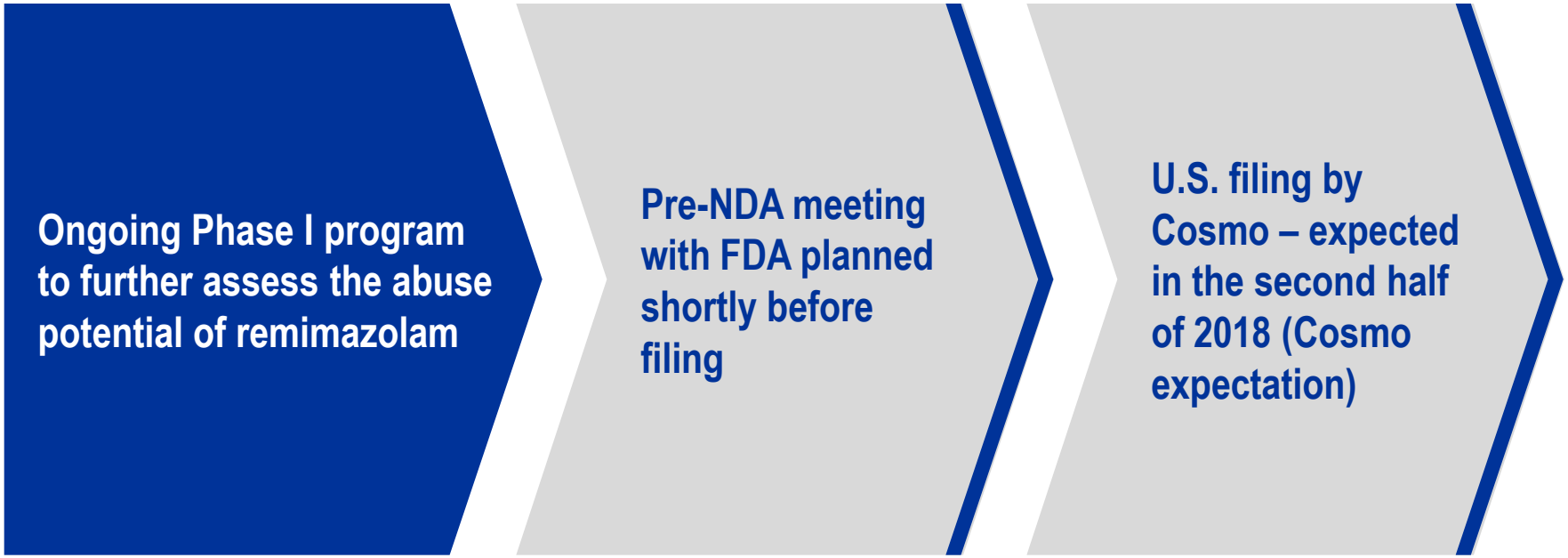
U.S. Phase pivotal Phase III study in colonoscopy: Supports business case (ability to treat more patients)

- With an average reduction of 20 min/procedure and an average number of procedures of 10-20/day/doctor, centers could increase throughput significantly

Time from start of medication to start of procedure	minutes
Remimazolam – 5.0/2.5 mg	**4.1**
Midazolam – 1.75/1.0 mg (1.0/0.5 mg in the elderly and debilitated)	15.9

Time to end of procedure to fully alert	minutes
Remimazolam – 5.0/2.5 mg	**7.2**
Midazolam – 1.75/1.0 mg	15.7

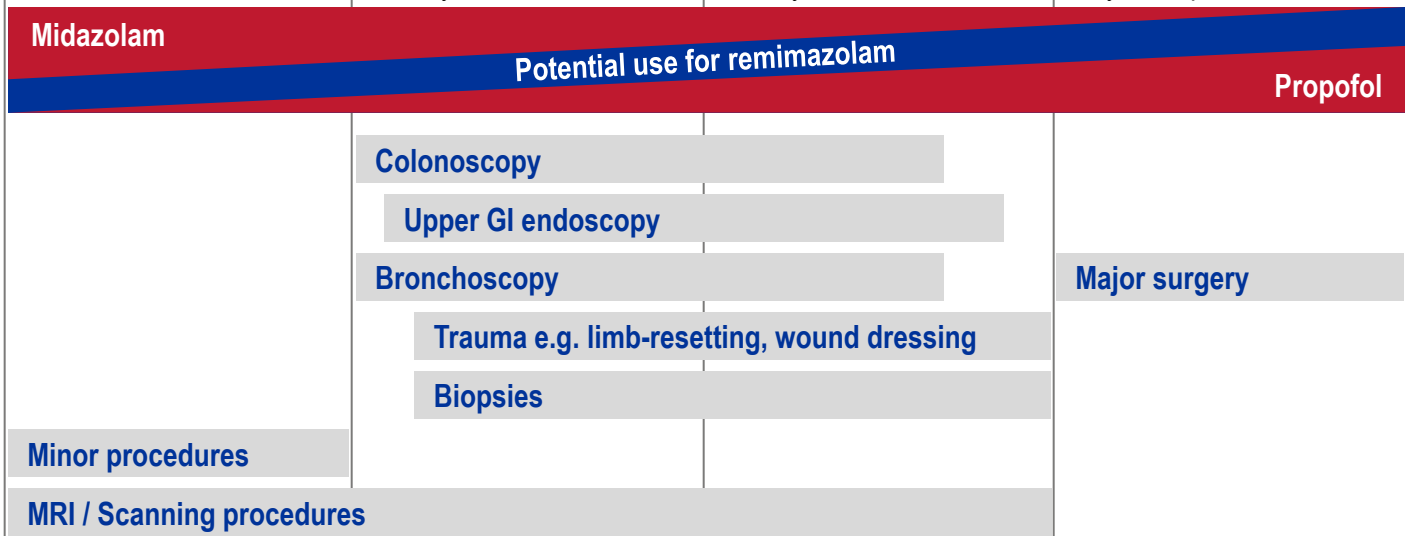
Remimazolam – Next Steps in the U.S.



Remimazolam – Multiple potential opportunities in sedation

Definition of general anesthesia and levels of sedation / analgesia (American Society of Anesthesiologists, 1999 – modified)

	Minimal sedation "anxiolysis"	**Moderate sedation / analgesia** "conscious sedation"	**Deep sedation / analgesia**	**General anesthesia**
Responsiveness	Normal response to verbal communication	Purposeful response to verbal or tactile stimulation	Purposeful response following repeated or painful stimulation	Unarousable even with painful stimulus
Airway	Unaffected	No intervention required	Intervention may be required	Intervention often required
Spontaneous Ventilation	Unaffected	Adequate	May be inadequate	Frequently inadequate
Cardiovascular function	Unaffected	Usually maintained	Usually maintained	May be impaired



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Remimazolam – Beyond the U.S. opportunity

Europe

- **Lead indication**: General anesthesia
- Ongoing Phase I study to support sample size calculation for a EU Phase III study in general anesthesia
- Solicit further guidance from European Medicines Agency (EMA) to determine components of new European development program
- Plan to commercialize remimazolam alone in the EU but also open to potential value-generating partnerships

Japan

- **Lead indication**: General anesthesia
- PAION has started to prepare filing of remimazolam in Japan – filing expected mid-2018, subject to further coordination with regulatory authorities and a potential partner
- Plan to partner in Japan during or after dossier preparation

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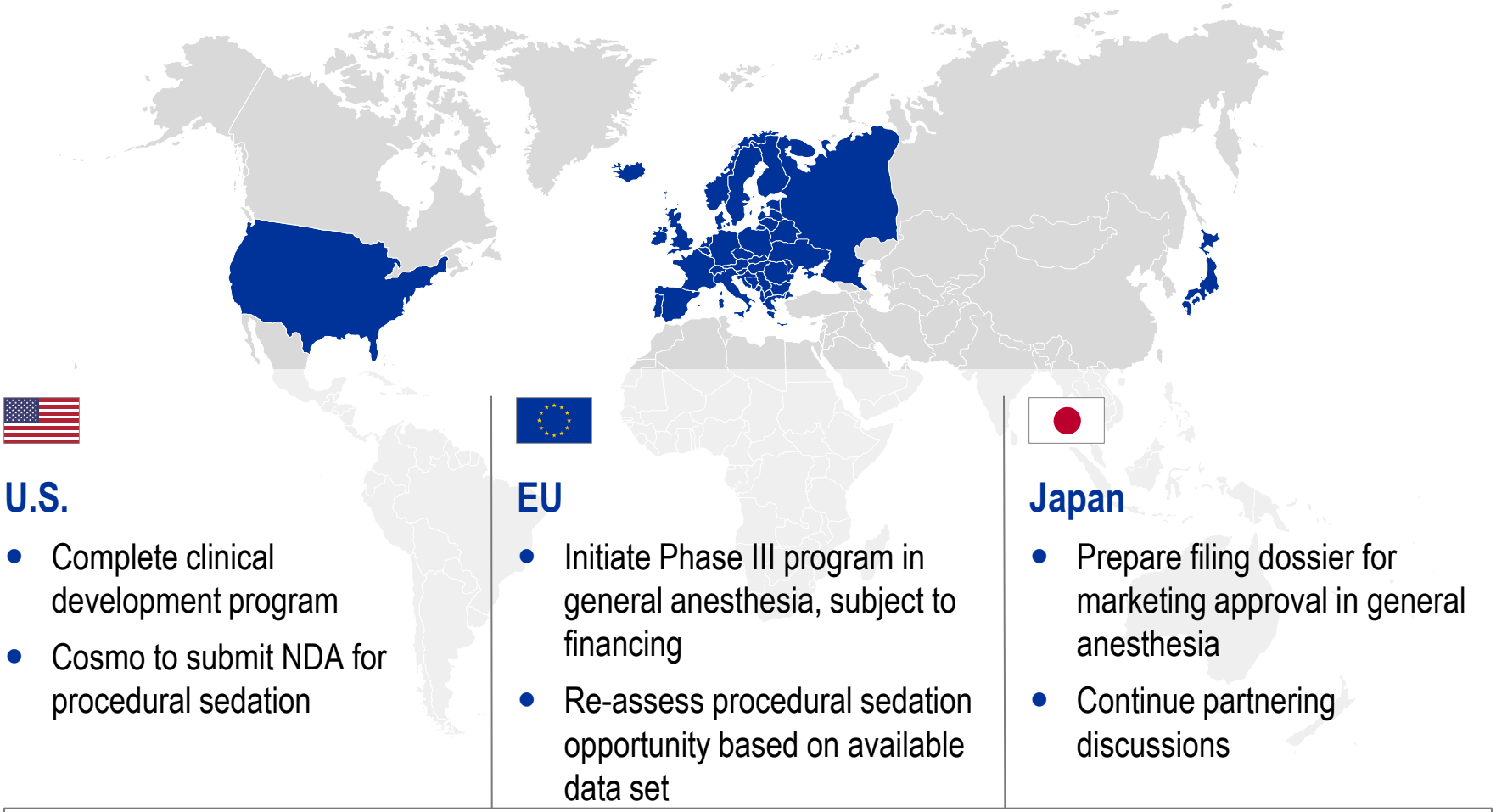
Outlook 2017 and 2018 – Driving remimazolam forward



U.S.

- Complete clinical development program
- Cosmo to submit NDA for procedural sedation

EU

- Initiate Phase III program in general anesthesia, subject to financing
- Re-assess procedural sedation opportunity based on available data set

Japan

- Prepare filing dossier for marketing approval in general anesthesia
- Continue partnering discussions

All partnered regions: Partners continue development and preparatory filing activities (Yichang Humanwell, Hana Pharm, R-Pharm, TR-Pharm, Pendopharm, Cosmo)

Remimazolam – A success story

2017	• Completion of U.S. Phase III program (ASA III/IV + bronchoscopy trial)
2016	• Cosmo becomes partner for remimazolam in the U.S. • Completion of U.S. Phase III colonoscopy trial
2015	• Start of the remimazolam Phase III programs in procedural sedation in the U.S. and general anesthesia in the EU
2014	• Capital measures of EUR 61 million secured for remimazolam Phase III programs
2013	• Japanese Phase III program with remimazolam successfully completed • Several license agreements with national champions in the anesthesia space (“String of pearls” strategy)
2012	• Sale of all Desmoteplase rights to Lundbeck (EUR 20 million) allows PAION to accelerate the development of remimazolam
2010	• PAION completes Phase II program in procedural sedation in the U.S.
2008	• PAION acquires CeNeS Pharmaceuticals plc with remimazolam
2007	• First Desmoteplase Phase III trial (DIAS 2) fails to meet primary endpoint, PAION identifies suitable patient population and successfully expands partnership with Lundbeck

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Q&A

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