Pharming Group NV

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Corporate Overview

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Pharming Group N.V. develops and commercializes human therapeutic proteins for innovative therapies meeting important unmet patient needs

- Founded in 1988
- Euronext: PHARM - market capitalization: €148m or $157m at €0.312
- Based in Leiden, Netherlands with approximately 105 employees
- 1st product approved in EU in 2010, US July 2014: RUCONEST®
  - Recombinant human C1-esterase inhibitor
  - For acute angioedema attacks in patients with hereditary angioedema (HAE)
  - Marketed in USA, EU and Israel now: US data exclusivity until 2026
- Lead development programs: Pompe and Fabry diseases, Hemophilia
- Collaboration with Sinopharm (CSIPI) for larger indications (e.g. Factor VIII)
- Good balance sheet and increasing revenues, with profitability expected in 2017

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Recent Corporate Highlights

RUCONEST® commercialisation

- Sales 9M 2016: €7m, including €5.8m from USA (30% of Valeant net sales)
- Paid $60 million upfront to Valeant for re- acquisition of North American commercialisation rights for RUCONEST on 7 Dec 2016
- Annual US sales run rate October/ November >$40 million
- US data exclusivity granted for 12 years until July 2026

Study of RUCONEST® for Prophylaxis of HAE

Phase 2 study (DBPC) met all endpoints in July 2016
Next stage being discussed with FDA
Prophylaxis market expected to grow to approximately 2 x Acute market
Total acute ($850m+) and prophylaxis ($700m+) rapidly approaching $2 billion

Building a pipeline beyond RUCONEST®

New pre-clinical programs for Factor VIII, Fabry and Pompe diseases
Uses rabbit founder technology
Combined market potential $4 billion+

Solid Financial Base

- December 2016: raised €90 million of net new debt and convertibles for financing of US deal
- Cash balance after US deal closing (08 Dec 2016) : €34 million
- Operating costs for 9M 2016: €15.1 million

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On 8 December, Pharming announced that it had completed the re- acquisition of the North American commercial rights to RUCONEST® from Valeant

- Deal value is $125 million, with an upfront of $60 million
- Self-funding milestones on sales up to a maximum of $65 million
- Pharming raised €95.6 million (net new debt & equity) on a market cap of €86 million
- Running US sales level at November 2016: over $40 million per year
- Main reason for deal is to enable proper development of RUCONEST® potential, leveraging our 10+ years experience of US HAE networks
- Commercial team all came to Pharming, with other key hires from the HAE world

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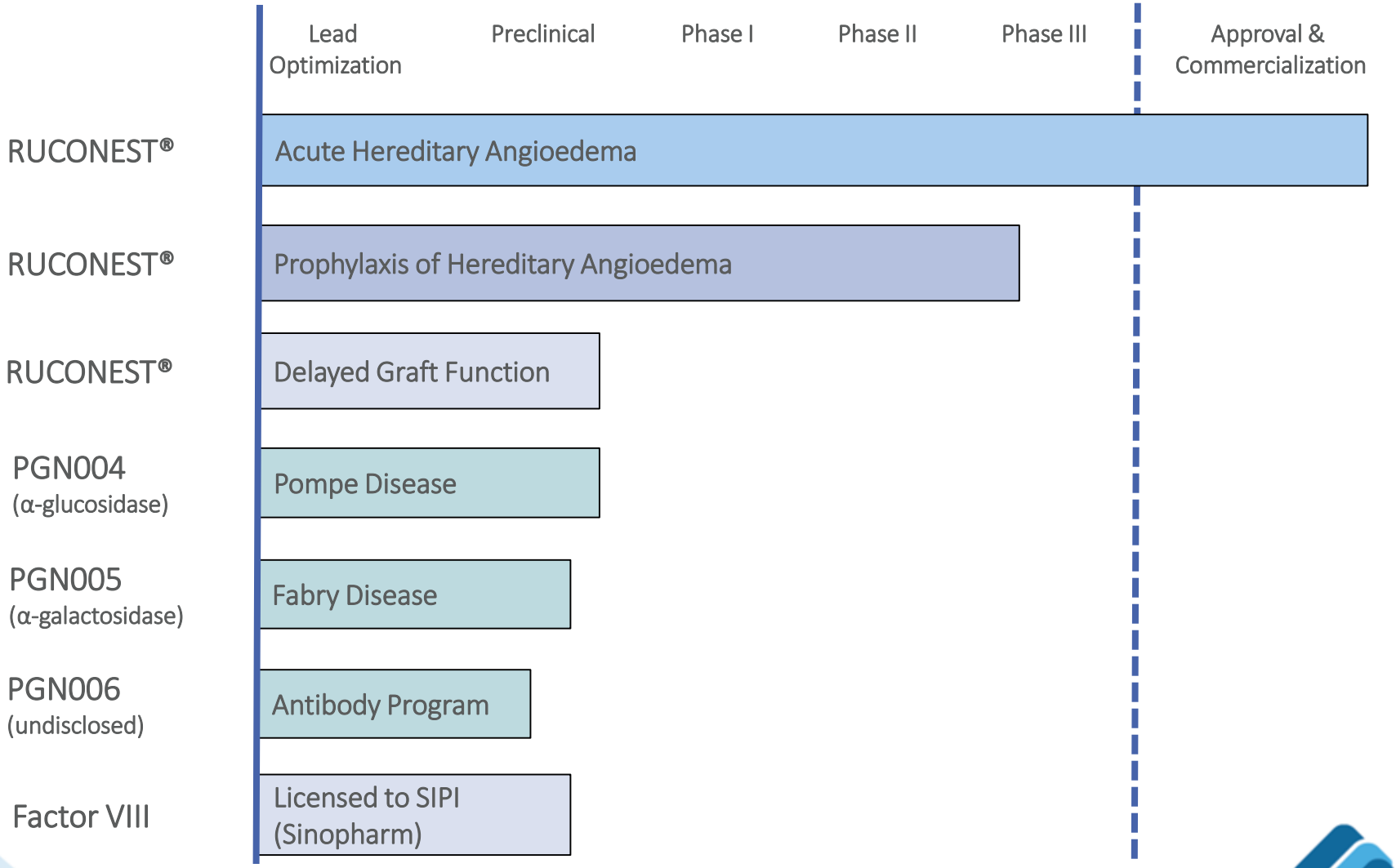
Pipeline



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Pipeline development

- France based research group for lead development/optimization and to enhance the rabbit technology further
- Initial internal pipeline projects in Preclinical Development stage
  - Focus on ERT treatments for Pompe disease, Fabry disease and Factor VIII for Haemophilia A (through Sinopharm collaboration with CSIPI)
  - Details of all programs will be released in a Research Day in Q2 2017
- Technology development
  - Improvements on platform (yields/ glycosylation)
  - Application of Technology Platform to other types/ classes of biologics
    - Antibodies and Complex Conjugates

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US HAE Market Features

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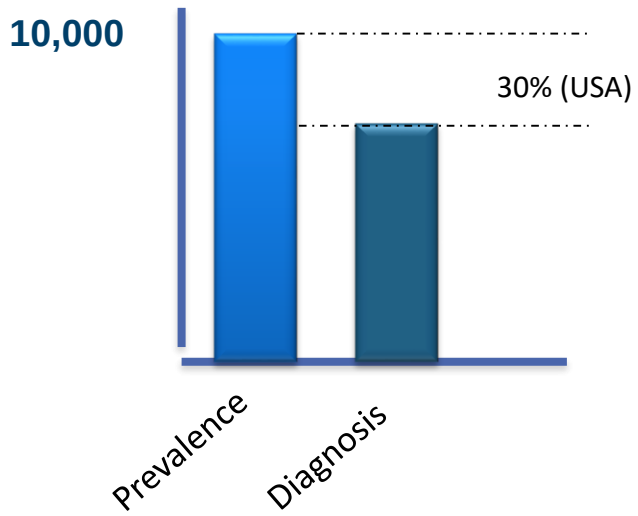
Hereditary Angioedema (HAE)

- Rare genetic disorder caused by mutations in the gene encoding C1-esterase inhibitor (C1INH)
  - Patients present with swelling, severe abdominal pain, or acute airway obstruction
  - Can be fatal if left untreated



- Prevalence estimates range from 1 in 10K–50K
- 75% of patients present prior to age 15
- Misdiagnosis is common; rate of diagnosis varies widely (US is most developed market)
- Growth comes from prescription growth and improved diagnosis
- Increase in attacks treated per patient per year
  - Still significant long term steroid prophylaxis
    - Ineffective, with high liver toxicity risk
  - Laryngeal attacks are potentially fatal
  - More aggressive treatment of all type of attacks

Inhibition of C1 esterase is Gold Standard for HAE treatment (protein replacement)

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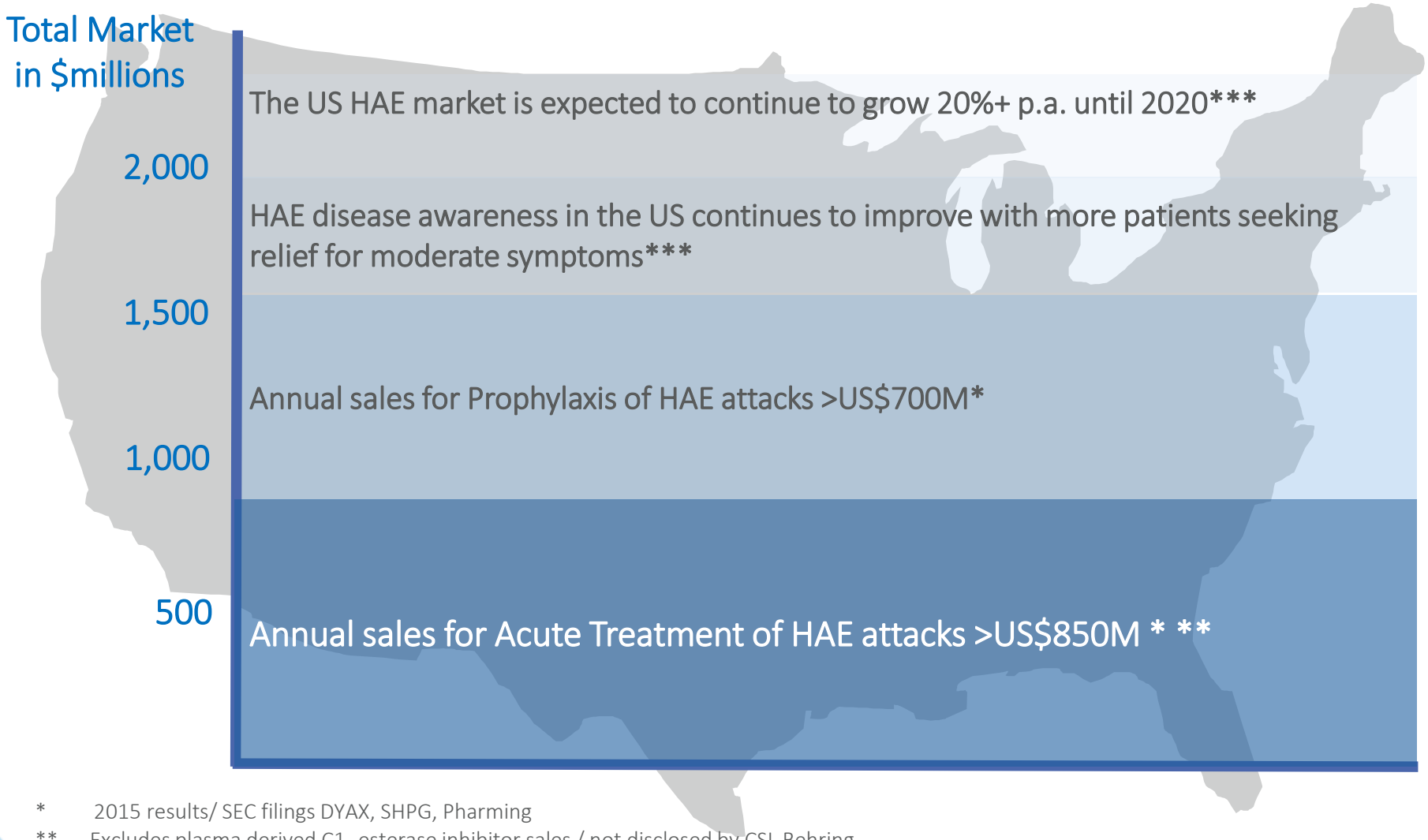
US HAE market: Rapid Growth, Significant Potential



* 2015 results/ SEC filings DYAX, SHPG, Pharming

** Excludes plasma derived C1- esterase inhibitor sales / not disclosed by CSL Behring

*** Leerink Swann, competitor interviews, 13 September 2012

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HAE Treatment Options

		Recombinant C1 Inhibitor	Plasma-derived C1 Inhibitor concentrates		Bradykinin receptor antagonist	Kallikrein inhibitor
Names		RUCONEST® ^	Cinryze^^^	Berinert	Firazyr**	Kalbitor^^^^
Owner		Pharming	Shire	CSL Behring	Shire	Shire
Sales 2016†		$40m	$680m ††	$250m	$579m	$52m
Efficacy		Good & consistent	Good	Good	Good	Good
	Dosing (C1INH)	50 U/kg*	~ 12 U/kg	20 U/kg	N/A	N/A
	Treatment type	Acute/Prophylaxis	Prophylaxis	Acute****	Acute	Acute
	Response < 4h†	89-96%	~ 52%	70%	58-74%	73%
Safety concerns		Very low risk of allergic reaction	Warning: Risk of blood clots	Warning: Risk of blood clots	97% injection site reactions	Black box warning: 3.9% Anaphylaxis
	Plasma risk	NO	YES	YES	N/A	N/A
Purity (C1INH)		>99.9%	±80%	±95%		
Relapse / worsening		Uncommon	Uncommon	Uncommon	11-31%***	17%
Administration		IV (SC, IM coming)	Twice weekly IV	IV	SC	SC (Hospital only)

Potential New Products

Clinical Trial	Under approval
Kallikrein inhibitor antibody	Plasma-derived C1 inhibitor concentrate
DX 2930 (a.k.a. SHP643)	Haegarda
Shire	CSL Behring
Phase III	FDA Review
	Good & consistent
N/A	80 U/kg
Prophylaxis – no acute	Prophylaxis – no acute
??	96%
Data to date is in mild patients only	Side effects not clear yet
N/A	High Plasma exposure
??	Uncommon
SC	Very slow large SC injection

† Sales figures are Pharming estimates based on run rate of most recent relevant selling company's releases and financial reports as well as IMS data and other proprietary databases. Response < 4h figures are for acute products, or represent reduction of attacks from base rate for prophylactic products

* Optimal efficacy of C1INH therapy is achieved at doses ≥50 U/kg ("Target levels of functional C1-inhibitor in hereditary angioedema". Allergy, C. E. Hack, A. Relan, E. S. van Amersfoort & M. Cicardi)

** Icatibant Clinical Briefing Document, CDER, FDA, 2011./ Aberer, et al. Ann Allergy Asthma Immunol 2010; 105(5):P238

*** Cicardi et al, N Engl J Med 2010;363:532-41.; Aberer, et al. Ann Allergy Asthma Immunol 2010; 105(5):P238; Lumry, et al. Ann Allergy Asthma Immunol. 2011;107:529 –537.

**** Berinert not licensed for peripheral attacks in the US.

^ Ruconest approved in US, EU and Israel, ^^Ruconest filed for laryngeal attacks (US) and will file for prophylaxis - reduces attacks by 74% in prophylaxis Phase II data, ^^^Cinryze not licensed for acute therapy in US. ^^^^Kalbitor not approved in EU.

?? Kalbitor moderate response rate is likely to be pathway-related, at least in part. Relapse rate is also likely to be pathway-related in part. Accordingly DX 2930 may also have these issues. In addition, the safety consequences of chronically inhibiting the contact pathway have not been studied, and this may also be a factor. Antibodies tend not to have large (>75%) response rates.

Note: New forms of products for different routes of administration may require clinical development and will require regulatory approval.

†† Shire had temporary failure to supply Cinryze due to contract manufacture problems in Q4 2016 and Q1 2017

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RUCONEST® starting to build sales

- Existing acute therapies have significant short-comings:
  - Inconsistent response/ efficacy and break-through attacks (up to 30%, requiring 2nd or 3rd dose)
  - Lower purity: Prone to cause both local and systemic side effects
  - Low dosing: Do not reach normal C1INH level at C Max
  - Plasma sourced: Infectious agents possibly transferred in manufacturing process
  - Plasma-derived C1 inhibitors carry warning for blood clots (Cinryze®, Berinert®)
  - Comparing published data: All competitors have lower reported response rates
- 80% of patients in US are treated by just 245 physician clinics
  - All directly targeted by Pharming, although most sales to date from outside group
  - Patients are very well organized (HAEA) and often play major roles in treatment decisions
- Long peak sales period
  - 12 Year exclusivity (until 2026) means that as it reaches peak sales, RUCONEST will have an extra 5-7 years without biosimilar competition from other recombinant C1 inhibitors
  - Sales growing in EU and RoW (Partnerships, direct sales and HAEi-GAP)

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Next indication in development: Prophylaxis of HAE

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RUCONEST® - Prophylaxis of HAE

- Phase II (double blind, placebo controlled, cross- over design) results meet primary endpoints for once and twice weekly regimen and show that twice-weekly prophylaxis treatment significantly (-72%) reduces attack frequency and features a 96% response rate (>50% reduction of attack frequency)
- The only approved product, a blood plasma derived C1- inhibitor concentrate dosed twice weekly reduces attacks by 52% and has a 50% response rate*
- RUCONEST® also approved for acute attacks, and so can become its own rescue therapy

		Placebo	RUCONEST®	RUCONEST®
Intent –to-Treat Analysis			Once/ week	Twice/ week
(n=32)	Primary: Mean number of attacks	7.2	4.4	2.7
	Reduction in attacks	-	39%	63%
	p-value		*0.0004*	*p<0.0001*
(n=31)	Secondary: % Patients with more than 50 % reduction in attack frequency		42%	74%
Per Protocol Analysis				
(n=23)	Mean number of attacks	7.5	3.8	2
	Reduction in attacks	-	49%	73%
	p-value		*p<0.0001*	*p<0.0001*
(n=23)	% Patients with more than 50 % reduction in attack frequency		57%	96%

*Zuraw et al; Nanofiltered C1- inhibitor concentrate for the treatment of HAE: NEJM 363;6 (August 2010): pp 513-522

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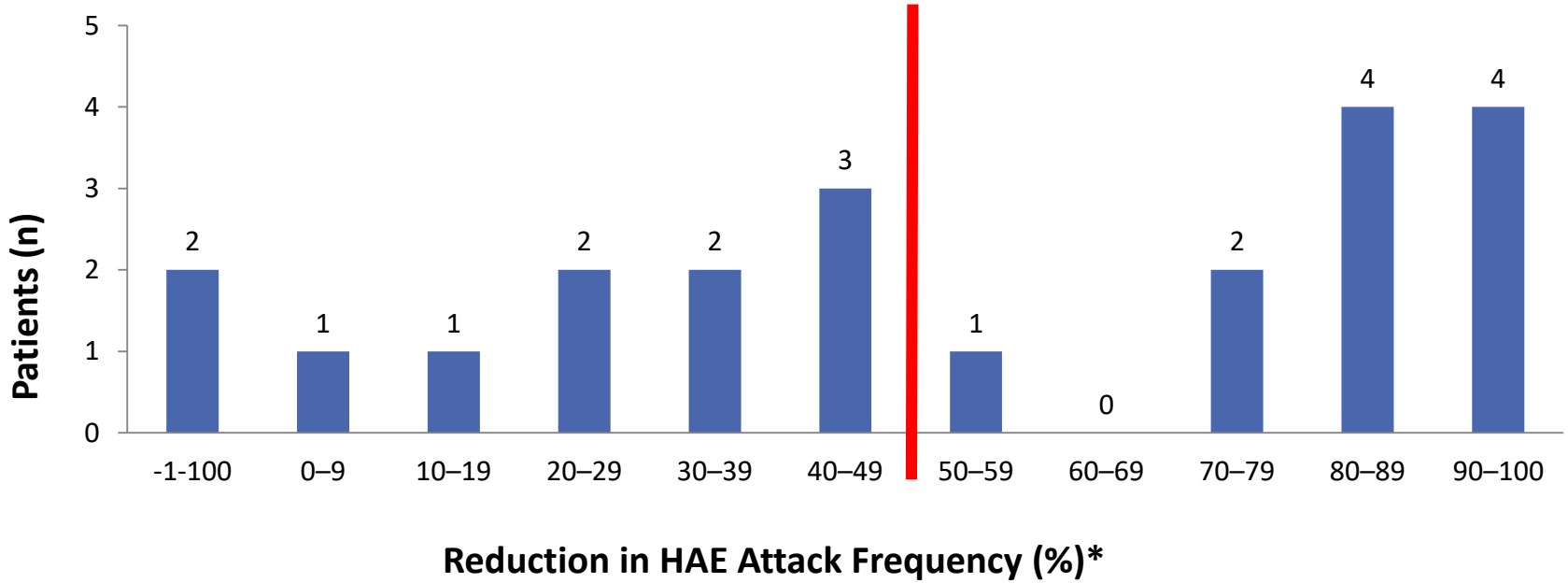
pdC1INH Prophylaxis: Clinical Response with Twice Weekly Dosing

Prophylaxis with Twice Weekly nano-filtered pdC1INH (n=22) resulted in varying reduction of HAE attack frequency



***2 patients had an increase in HAE attack frequency while receiving nanofiltered C1INH prophylaxis: One patient an increase of 8% and one patient an increase of 85%.**

C1INH = C1 esterase inhibitor; HAE = hereditary angioedema.

FDA Briefing Document. Blood Products Advisory Committee Meeting. http://www.fda.gov/ohrms/dockets/ac/08/briefing/2008-4355B2-1b.htm. Published May 2008. Accessed July 26, 2016.

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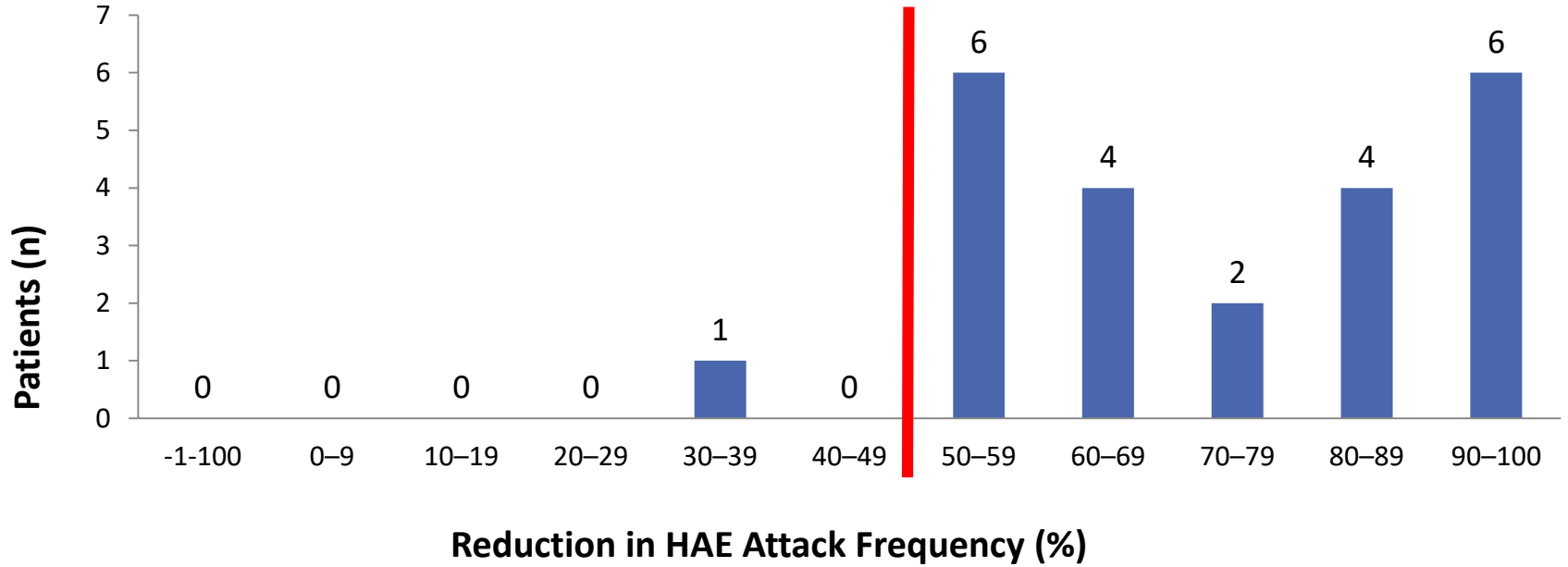
rhC1INH Prophylaxis: Clinical Response With Twice Weekly Dosing

Prophylaxis with Twice Weekly rhC1INH resulted in consistent reduction of HAE attack frequency (n=23)



HAE = hereditary angioedema; rhC1INH = recombinant human C1 esterase inhibitor.

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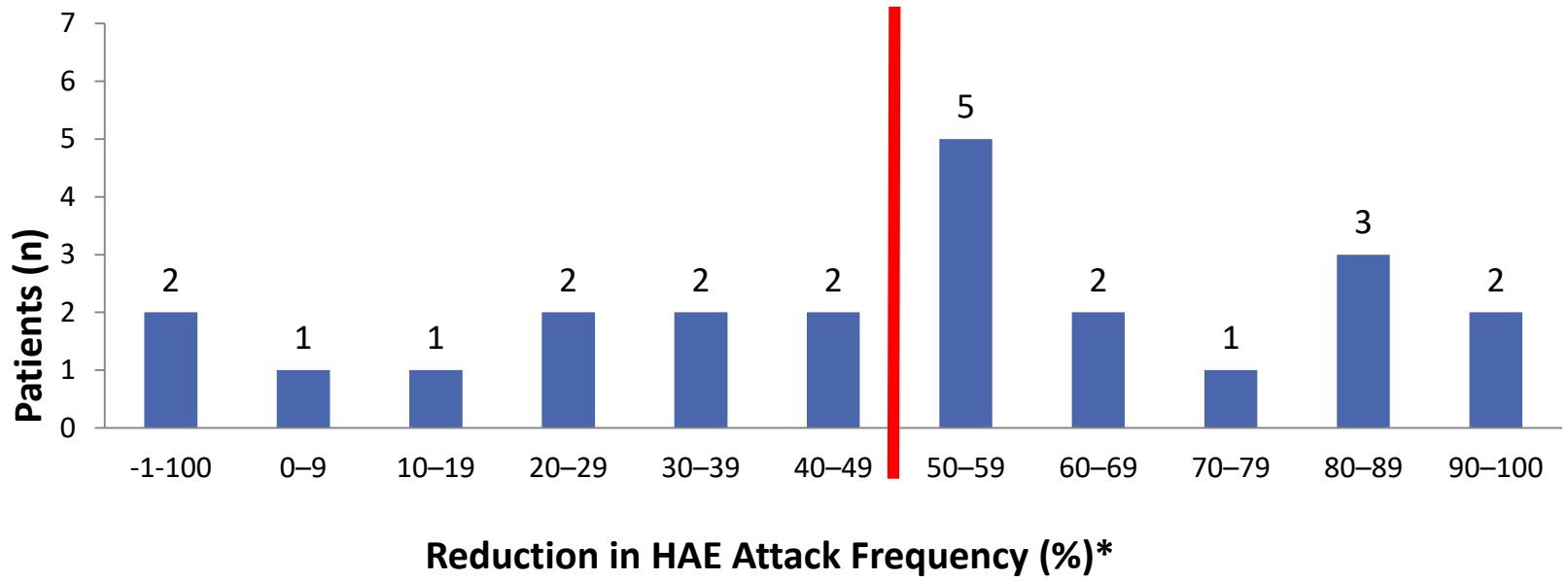
rhC1INH Prophylaxis: Clinical Response With Once Weekly Dosing

Prophylaxis with Once Weekly rhC1INH (n=23) resulted in varying reduction of HAE attack frequency



***2 patients had an increase in HAE attack frequency while receiving once weekly rhC1INH prophylaxis. One patient an increase of 40% and one patient an increase of 62.5%**

HAE = hereditary angioedema; rhC1INH = recombinant human C1 esterase inhibitor.

Data on file. Pharming. 2016.

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Re- acquisition of North American Commercialisation rights for RUCONEST Transaction Overview

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Commercial Plan Highlights

Operations and Governance

- Acquired entire Valeant team as part of transaction (n=11), now expanding
- Setting up MSL and patient services team
- Transition Services Agreement with Valeant in place; seamless transition
- Commercial Advisory Board to determine/monitor strategy in US, including former *Cinryze* executives
- Former *Firazyr* executive as Commercial Operations head
- RUCONEST SOLUTIONS total care plan for patients
- US office in New Jersey, including legal/ regulatory and field support team

Sales Development

- $24 million net sales in 2015 - mostly to early adopters
- Valeant reduced sales force from 24 reps in May 2015 to 8 reps in Oct 2015.
- Valeant 9M 2016 net sales just over $21 million
- Steadily increasing sales trend in 2016: Annualized sales rate on basis of Oct-Nov 2016: >$40 million

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RUCONEST hits the big issues:

Severity of Attacks

- RUCONEST® treats new attacks first time with one dose (89-96%) – much higher than next nearest competitor (comparing published data)

Frequency of Attacks

- RUCONEST® Phase II data showing 72% reduction in attack frequency and a response rate of 96% in moderate to severe patients when taken 2 x weekly

Willingness to accept Injection/IV

- RUCONEST® is being adapted to be delivered in a small concentrated injection, for future use as intramuscular (IM) injection and/or subcutaneous injection

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Short term extra opportunities to accelerate growth

- Direct sales contracts
  - Agreements under review for direct sales with potential to significantly increase patient base rapidly
    - New Buy-and-Bill customers
    - New Specialty Pharmacy customers who could not deal with Valeant
- Specific Geographic areas
  - Underserved Minority patient groups in several territories that are not being served adequately and who have expressed interest in recombinant therapy
- Extend Territory
  - File for approval in Canada and Mexico, either alone or with partners

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Attractive Growth Proposition

- RUCONEST® can be sold better by Pharming, which knows the market better and is willing to put greater and dedicated/ focused resources behind the sales team
- Pharming has an excellent reputation in the HAE space, and strong support from the patients‘ associations
- Pharming expects to be operationally profitable in 2017 even if sales should stay at current, end of 2016, levels
- With planned investment and significant expansion of MSLs and sales reps, we believe we can get very strong sales growth over the coming years
- This will produce positive Net Earnings at a significant level for Pharming after milestones and financing costs/repayment
- Our next products are expected to come online in 2020-2021 after development, and so we would have a US commercial operation ready for selling those immediately

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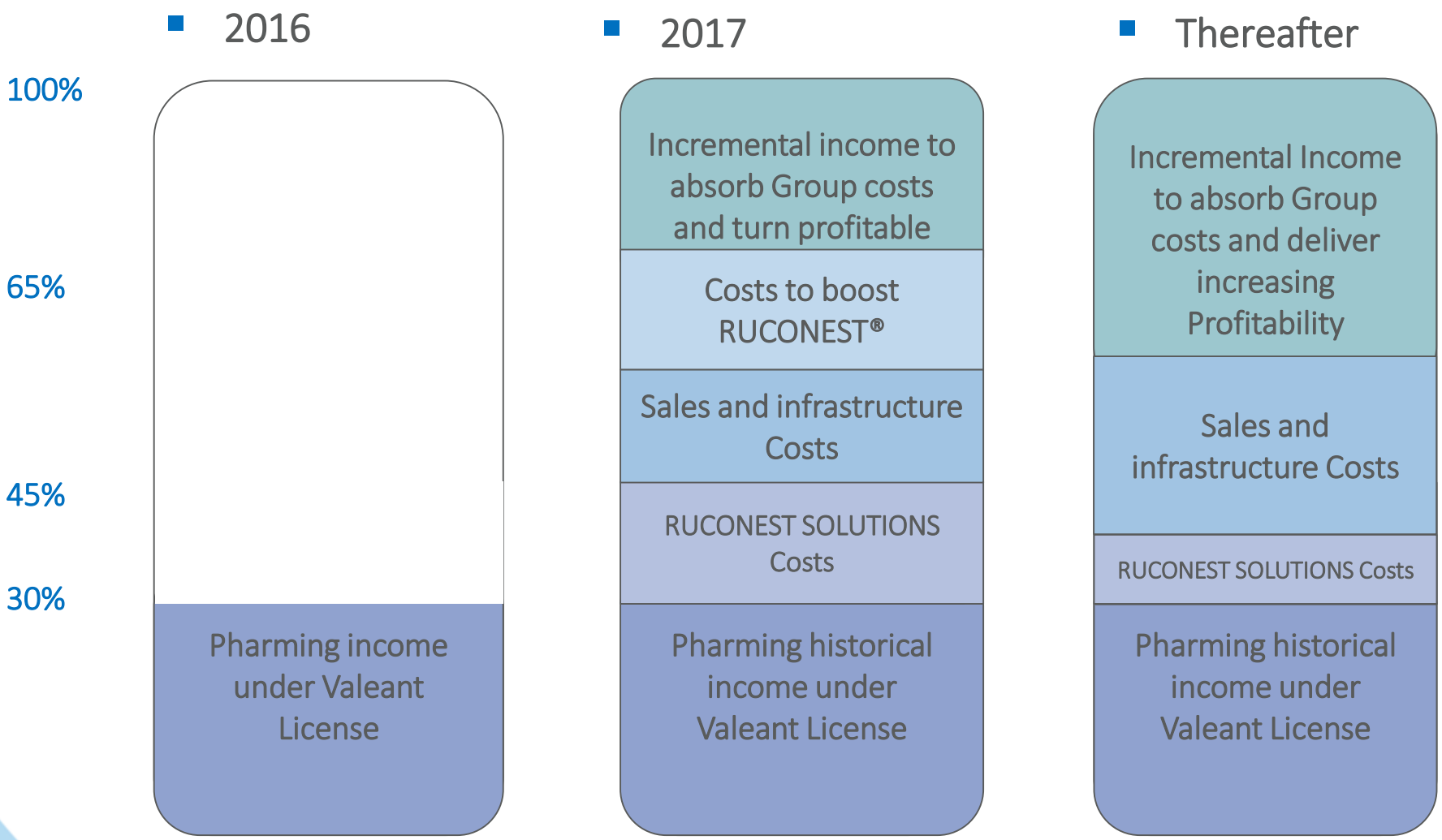
Financial impact



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Pro forma at Half-Year and 9M 2016*

Amounts in €m (unaudited) except per share data	Actual YTD 2016	*Pro Forma* YTD 2016	% Net Change	*Pro Forma* 1H 2016	% Net Change*
Income Statement					
Product sales	7.0	20.5	*193%*	12.4	*195%*
License fees	1.7	0.7	*(59%)*	1.1	-
Revenue	8.7	21.2	*144%*	13.5	*155%*
Gross Profit	5.5	18.0	*228%*	11.5	*248%*
Other (non-product) income	0.3	0.3	-		
Costs	(15.1)	(21.4)	*(42%)*	(14.7)	*52%*
Operating Result	(9.4)	(3.1)	*67%*	(3.1)	*50%*
Balance Sheet					
Cash & marketable securities	17.0	23.7	*39%*	26.3	*21%*
Share Information					
Earnings per share	(0.025)	(0.010)	*39%*	(0.013)	*19%*

* For comparison and illustrative purposes only; on the basis that the Valeant transaction had been completed as at 01 January 2016

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Back- up slides

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rhC1INH for HAE Prophylaxis: Study Design

- Phase 2, double-blind, multicenter, cross-over study
- Patient population:
  - (n=32) ≥13 years of age
  - Functional C1INH <50% of normal
  - Frequent HAE attacks*
- Patients are randomized to 3 separate 4-week treatment periods, separated by 1 week and all undergo the 3 different treatment periods
  - rhC1INH 50 IU/kg† twice weekly
  - rhC1INH 50 IU/kg† once weekly + placebo once weekly
  - Placebo twice weekly
- Rescue medications permitted for breakthrough attacks‡
  - pdC1INH only allowed for laryngeal attacks

*≥4 attacks per month.
†50 IU/kg for patients <84 kg; 4200 IU for patients ≥84 kg.
‡Rescue medications could include open-label rhC1INH, HAE specific medications (eg, icatibant) or symptomatic medications (narcotics).
HAE = hereditary angioedema.; rhC11NH = recombinant human C1 esterase inhibitor.

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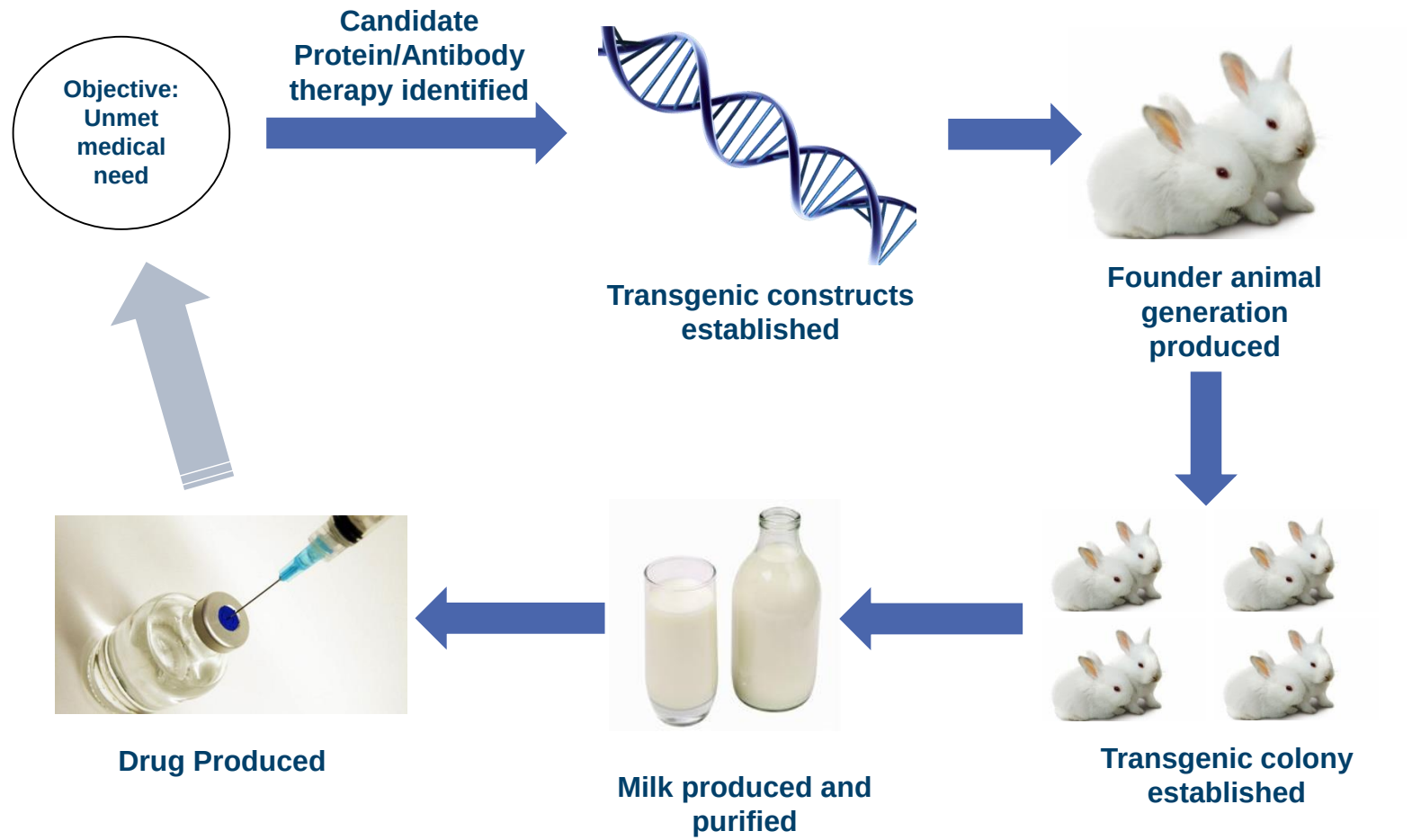
Pharming: Protein or antibody drugs produced through transgenic animal milk, with high efficacy and low immunogenicity:



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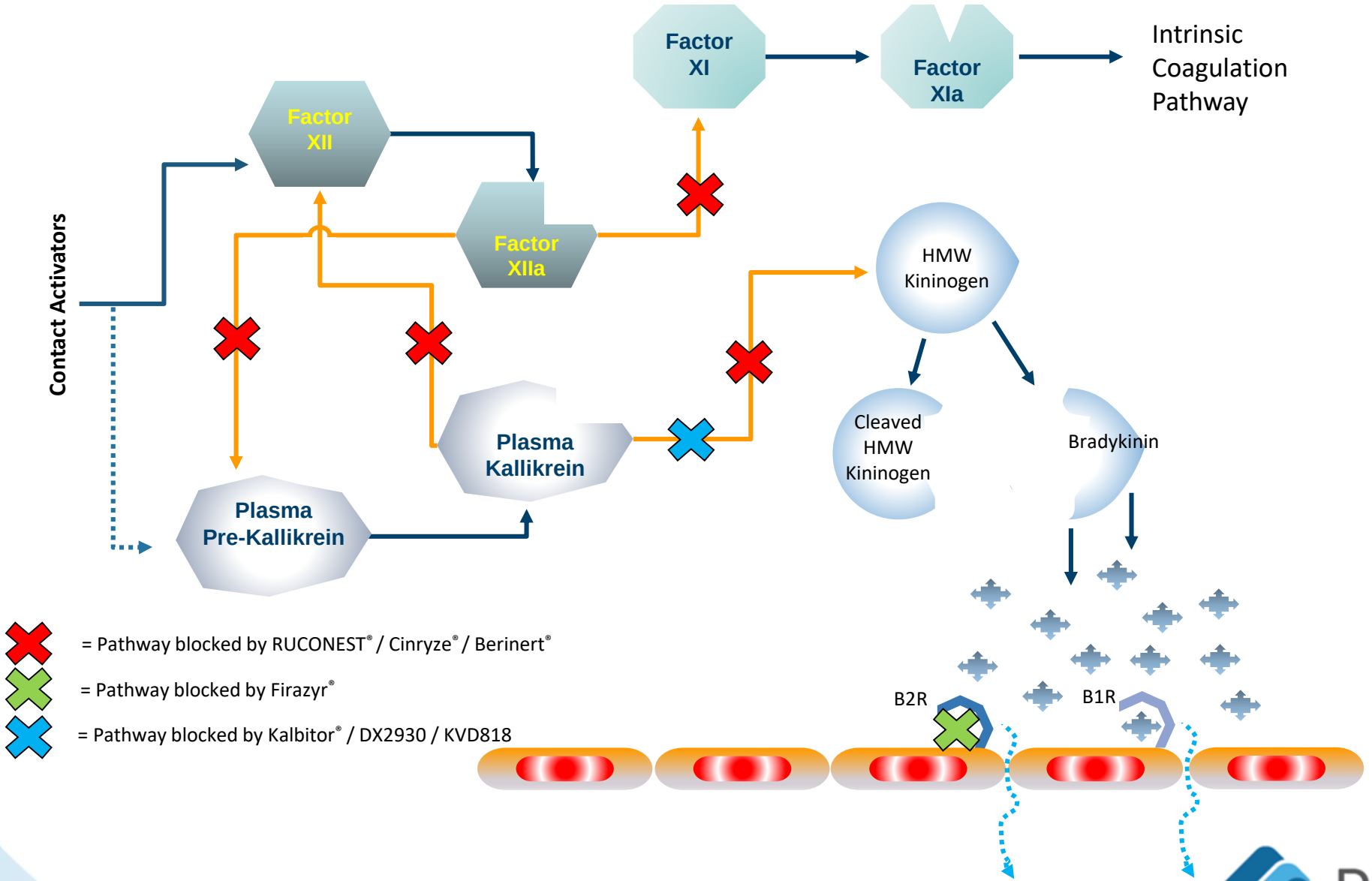
Therapy Effects



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