Pharming Group NV
Biocapital Europe

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Sijmen de Vries, CEO

HEALTH ■ SCIENCE ■ PRODUCTS

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Strategy & expertise

- To develop innovative products for diseases with high unmet medical needs
  - Our commercial focus is primarily the specialty pharmaceutical market
  - Pharming covers the entire value chain through internal expertise and external collaborations
- Proprietary technology (transgenic) platform for protein production
  - Versatile & scalable without typical bio-reactor up-scaling risks
  - Strong IP and know how protection
  - Aim to leverage the inherent value of this platform
- Current focus on recombinant C1-inhibitor (Ruconest™/ Rhucin®) franchise
  - Initial indication: acute treatment of hereditary angioedema (HAE) attacks
  - Additional indications
    - Antibody Mediated Rejection (Kidney Transplant)
    - Ischemia Reperfusion Injury (Delayed Graft Function, Acute Myocardial Infarction)

Pipeline

	Indication	R&D	Pre Clinical	Phase I	Phase II	Phase III	Registration	Market
Ruconest™ / Rhucin®								
Ruconest™ (rhC1INH) (Europe)	Hereditary Angioedema							
Rhucin® (rhC1INH) (US)	Hereditary Angioedema							
rhC1INH additional indications								
rhC1INH	Antibody Mediated Rejection (Kidney)							
rhC1INH	Delayed Graft Function (Kidney)							
rhC1INH	Acute Myocardial Infarction							
Other Recombinant Products								
rhFibrinogen	Fibrinogen deficiency							
rhCollagen	Tissue repair							
hLactoferrin	Nutritional applications							

Core focus products/indications

Partnerships + risk sharing models for further development

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HAE & C1 inhibition

- Rare genetic disorder caused by mutations in the gene encoding C1 esterase inhibitor (C1-INH)
  - Low functional levels of the complement control plasma protein C1-INH
  - Patients present with swelling, severe abdominal pain, or acute airway obstruction
- Prevalence of disease estimated at 1 in 30,000
- 8+ swelling episodes requiring treatment per patient per year
  - Despite wide spread long term steroid prophylaxis
  - Laryngeal attacks are potentially lethal
  - Significant Quality of Life issues for patients given frequency of attacks

- Three systems active in HAE (Complement, Contact, Fibrinolytic)
  - C1 inhibitor (missing protein) controls all three systems

- Treatment with C1 inhibitor considered ‘gold standard’ by clinicians

HAE Treatment - C1 inhibitor

Complement System

C1 → C1a → C4a3a5a

C1INH is a key regulatory suppressor of the complement, coagulation, and kallikreinkinin cascades

Contact System

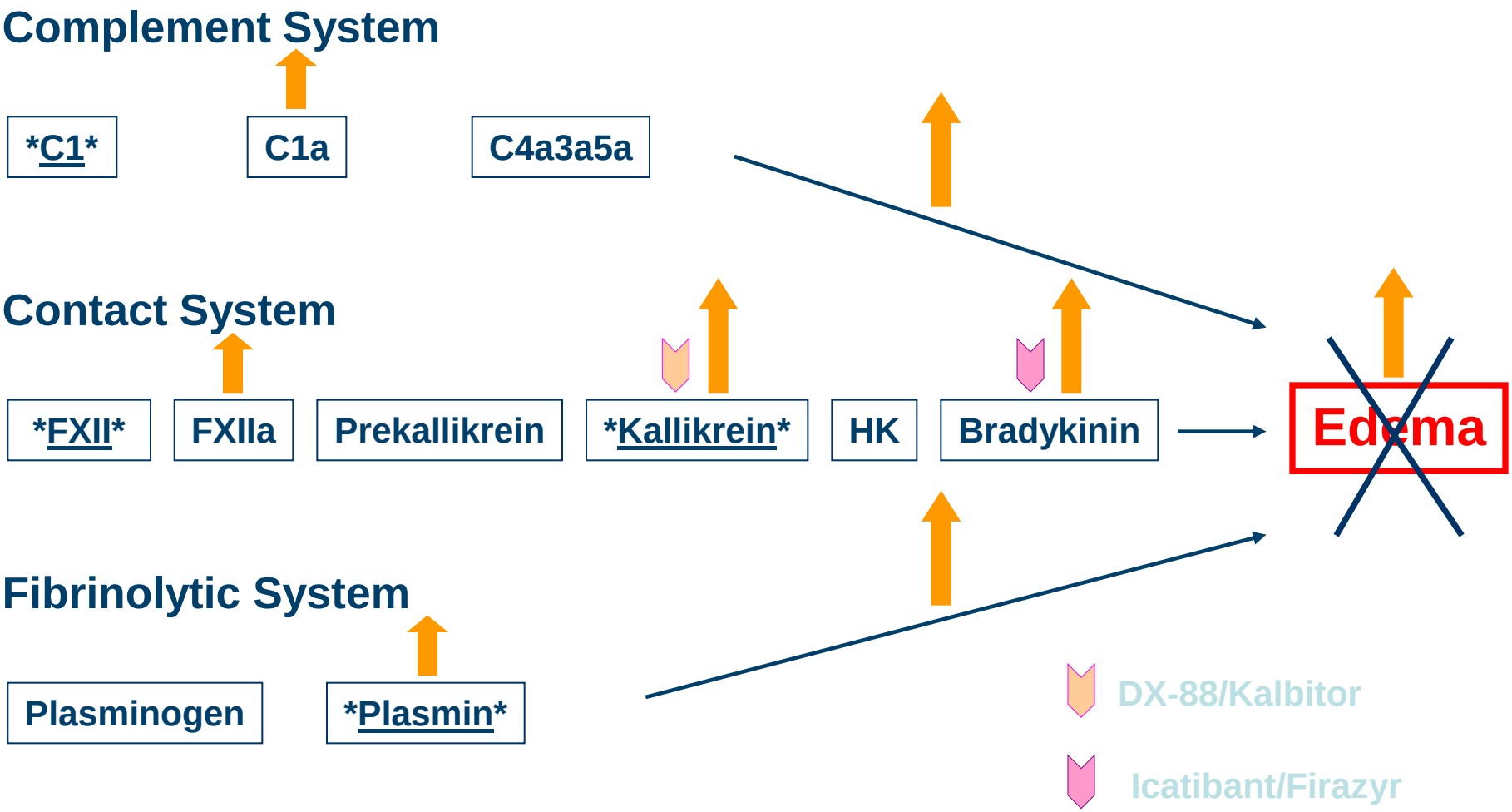
FXII → FXIIa → Prekallikrein → Kallikrein → HK → Bradykinin → Edema

Fibrinolytic System

Plasminogen → Plasmin

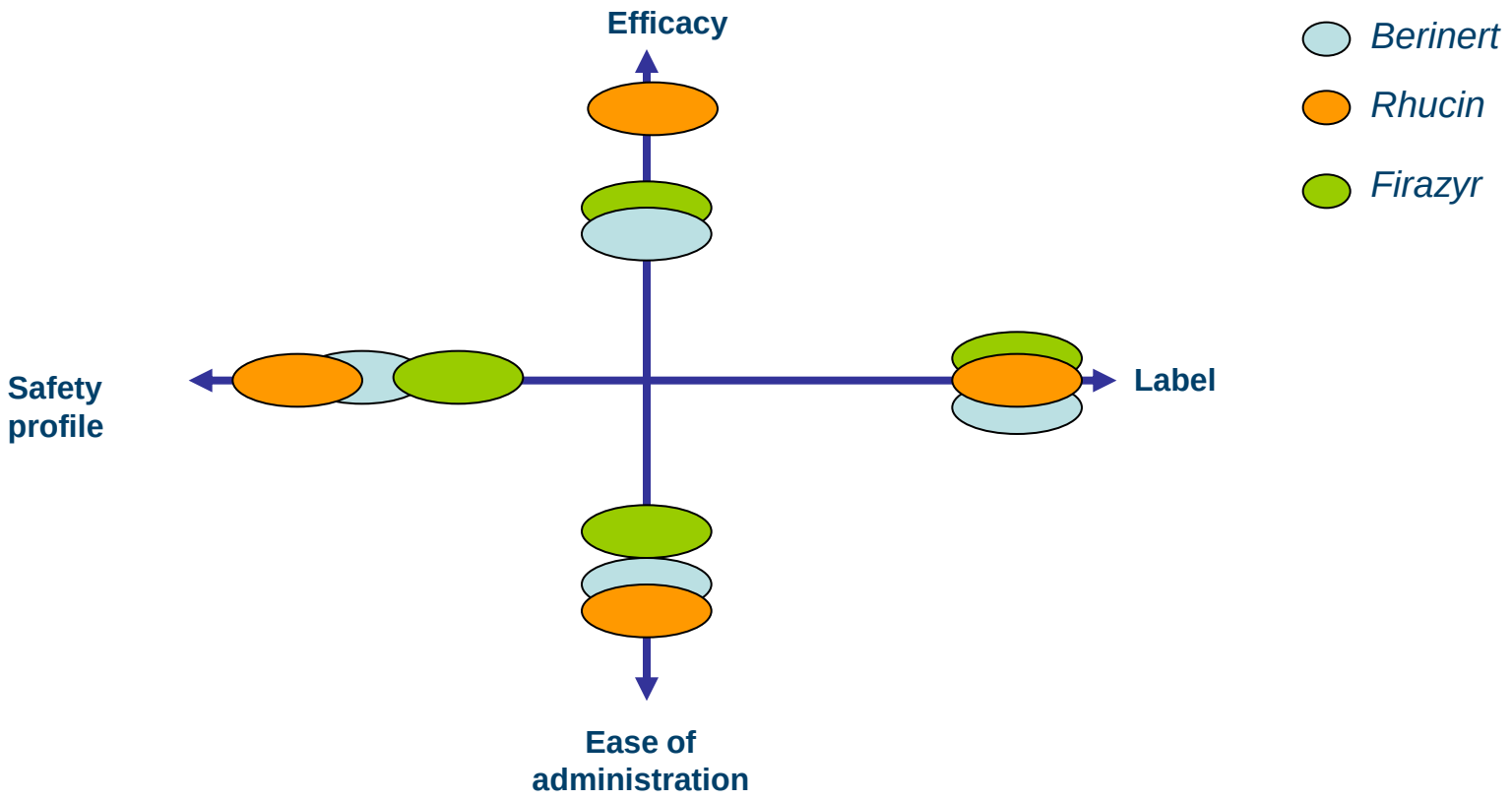
C1INH

Pharmacology of the competition



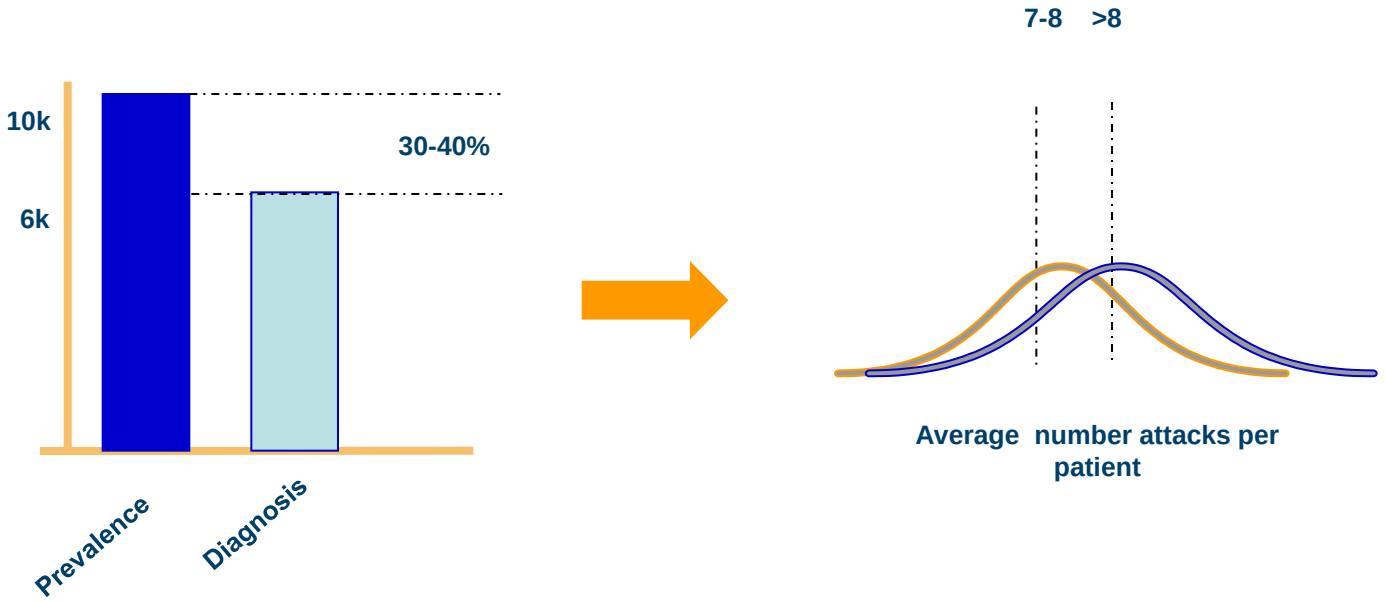
__ - Sites inhibited by C1INH

Potential positioning: EU (Acute)



- **Berinert: widely used in EU, under-dosed (500-1500 U)**
- **Firazyr (EU): breakthrough attacks, local injection reactions, pharmacology undermines efficacy**

Market Drivers: Patient numbers



The prevalence rate is difficult to ascertain and ranges from 1 in 10K–50K individuals with 75% of patients presenting with symptoms before age 15. Misdiagnosis is also common.

Estimates suggest that the US & EU each have ~10,000 patients, with >6,500 patients seeking treatment in US, each having ~8 moderate/severe attacks per year.

As more therapies come to market for HAE, recognition by physicians should increase, helping to grow the treatable patient population.

Cash value increasing

1. Increasing diagnosis/physician awareness
2. Patients seeking treatment even for moderate symptoms
3. New drug pricing

EU: EUR 2000-3400 per attack
US: USD 5000-7950 per attack

Steroids and historical/ initial PD C1 inhibitors → New targeted therapeutics

Market size	Market size
EU: € 110 m	Significant market expansion
US: $ 150 m +	C $1bn WW

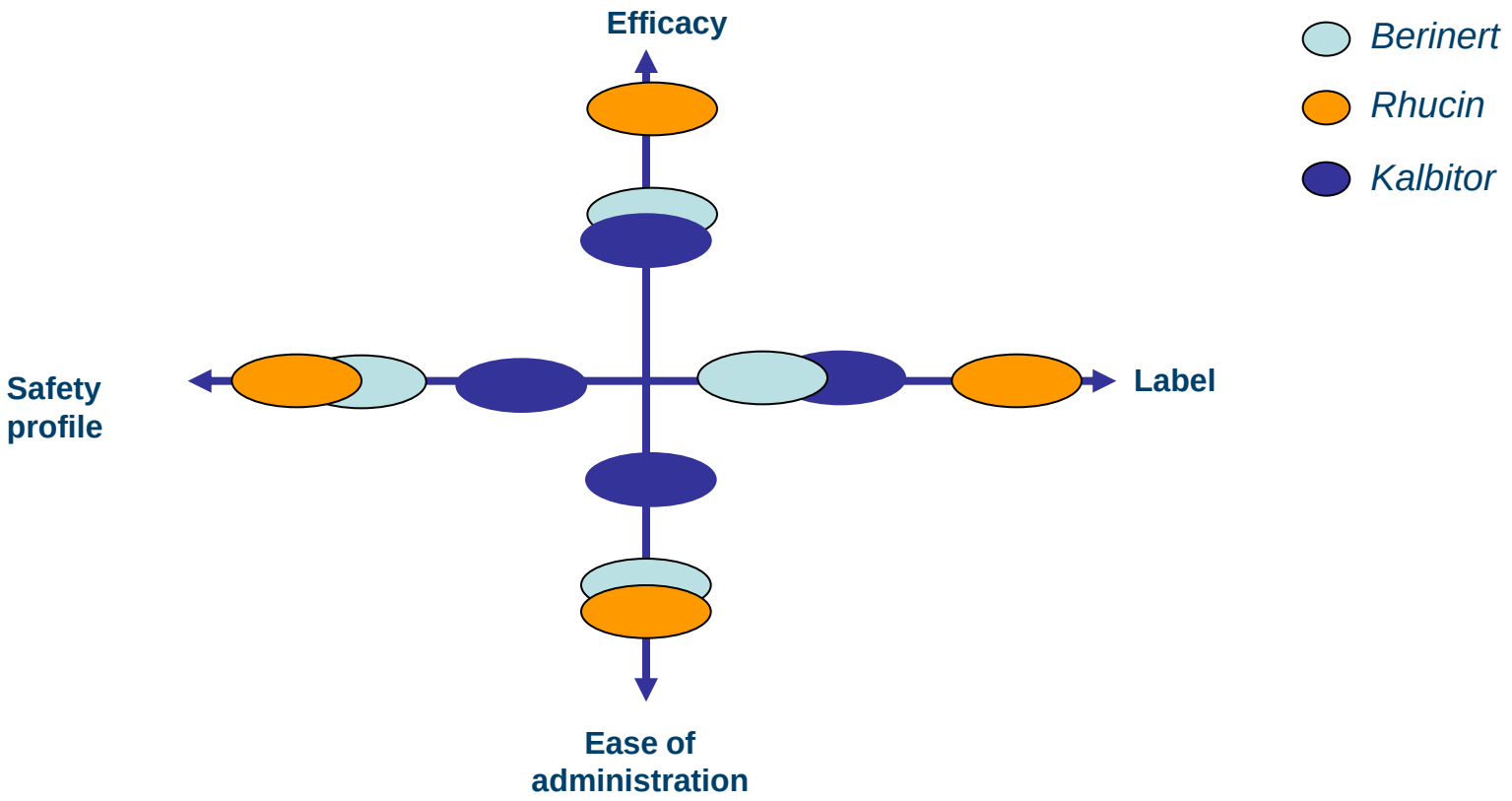
EU Commercialisation Strategy

- Distribution partnership with Swedish Orphan Biovitrum (SOBI) for Ruconest™ in 24 EU markets + Norway, Iceland, Switzerland
- Partnership delivered € 8m in milestones and is expected to deliver (significant) cash flows from proceeds of (future) sales
- Option to participate in (costs and benefits) development of future follow-on indications
- SOBI is a strong regional partner with focus on orphan diseases
- Ruconest will be a key growth driver for SOBI
- First sales achieved in Dec 2010
  - Roll out continues across Europe through 2011
- Additional distribution agreements in place for:
  - Spain, Portugal and Greece with Esteve

US Commercialisation Strategy

- Commercialisation agreement with Santarus for North America
  - Excellent specialty pharmaceutical partner
  - Small specialty sales force can reach large proportion of prescribers
  - Small target core audience of prescribers/ initiators (~1000) to be covered by specialty field force of approximately 25.
- US$15m upfront received upon signing
  - Additional US$35m in clinical regulatory and commercial milestones
  - Potentially a further US$45m in sales level-related milestones
- Discussion with FDA scheduled to discuss BLA submission process
- Sharing costs and benefits of development and commercialisation of follow-on indications

Potential positioning: US (Acute)



- Kalbitor: Black box warning on anaphylaxis on label, admin is difficult, pharmacology undermines efficacy
- Berinert: Incomplete label (no peripheral and laryngeal attacks)
- Cinryze: Not approved for acute, insufficient evidence for efficacy/ prophylaxis only

Prophylaxis: OPERA (exploratory) study

- Twenty five HAE patients with a history of frequent attacks received weekly administrations of 50U/kg of Ruconest for 8 weeks.
- Patients reported a median of 60 HAE attacks (range 39 to 467) over the past two years
  - corresponding to an average of 1 attack every 12 days (*ranging from once every 2 days to once every 18 days*).
- The breakthrough attack rate observed during the study was much lower with a median of 2 attacks over the 8 week period
  - corresponding to an average of 1 attack every 28 days (*ranging from no attacks to once every 5 days*).
- Weekly administrations of 50 U/kg rhC1INH was generally safe and well tolerated.

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Maximizing Value Rec. C1 inhibitor Franchise

Two indications for immediate further development:

- Antibody Mediated Rejection (AMR): rejection after organ transplantation resulting in damage that can lead to organ failure
  - Circulating donor specific Abs leading to complement activation and cell death

- Delayed Graft Function (DGF): most common complication after kidney transplantation
  - Ischaemia reperfusion injury

Antibody-Mediated Rejection (AMR)

- Prevent complement-mediated cell damage
- Pre-clinical studies:
  - Proof-of-concept study completed and published
    - Inhibition of rejection throughout duration of treatment with rhC1INH.
    - Follow-up study to start H1-2011
- Clinical trial:
  - Active IND
    - Initial clinical phase II trial initiated

Delayed Graft Function (DGF)

- An ischaemia/reperfusion indication:
  - Reperfusion accompanied by inflammatory response with complement activation
  - DGF hypothesized to reduce short and long term graft function
- Pre-clinical studies:
  - Proof-of-concept study with auto transplantation completed and published
    - Reduction in inflammatory response and tissue damage
  - Subsequent study with auto transplantation evaluating long-term protective effects to start H1-2011

Clinical trial:

  - IND submission and protocol preparation in 2011

Acute Myocardial Infarction (AMI)

- Another ischaemia/reperfusion injury (RI) indication
- Proof-of-concept obtained with plasma-derived C1INH in pre-clinical models and clinical studies
- Pre-clinical studies:
  - Exploratory study to start H1-2011
  - Proof-of-concept study to evaluate efficacy in myocardial infarction model expected to start H2-2011
- Clinical trial:
  - Submission of an IND after obtaining proof-of-concept with rhC1INH

Results 2010

- EU filing of Ruconest in September 2009 ✓
- EU distribution agreement for Ruconest ✓
- EU Positive opinion for Ruconest ✓
- US Partnership for Rhucin ✓
- EU launch for Ruconest ✓
- US BLA submission Rhucin ✓
- Debt restructuring ✓
- Equity Investment ✓

Financial Highlights

	YE'10	YE'09
Liquidity position (€M)	10.4*	2.3
Net cash used for operating activities (€M)	(3.2)**	(24.3)
Operating Loss (€M)	(44.1)^	(27.8)
Net loss (€M)	(50.2)^	32.1
Convertible debt (€M)	n/a	10.9
Number of shares outstanding	436,261,010	154,501,037

*** Excludes €10.0m receivable from Socius which was received in early January and March**

****Operating cash outflows decreased to €22.4m (2009: €28.0m), including € 2.9m non-recurring for DNage .**

^Includes significant non-cash impairment charges (liquidation of DNage)

Plan 2011

- EU roll out of Ruconest (SOBI) through 2011
  - Will take most of year as reimbursement is national
- Additional regional C1 Inhibitor licensing deals
  - Through 2011 to increase geographical coverage
- BLA filing process
  - Meet with FDA in coming weeks to discuss next steps
- Progress reperfusion injury programme (DGF/ AMI)
  - Data read outs on new indications in 2012
- Continuing focus on operational efficiencies
- Focus on non- dilutive sources of financing

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