

YOUR LINK TO THE FUTURE



ASCENDIS PHARMA

Company Presentation May 2010

COMPANY PROFILE

- Ascendis Pharma is an emerging Hi-Tech Specialty Pharma company with focus on endocrinology and CNS

- Ascendis' lead program - TransCon PEG hGH - is a superior growth hormone with a once-weekly dosing regime
 - Clinical stage program targeting EU and U.S. submission in 2013/2014

- Ascendis has six high-value products in its portfolio, e.g. once-weekly insulin product and once weekly insulin/exendin combination, as well as once-monthly paliperidone product

- Our innovative prodrug technology platform, TransCon, provides a sustainable pipeline for the future and strategic BD value

BACKGROUND

- Ascendis acquired Complex Biosystems in December 2007
 - TransCon technology invented and matured by Complex Biosystems since 2002

- Ascendis runs in a semi-virtual manner, utilizing strategic teams across Denmark, Germany, and the U.S. Ascendis currently has 25 full-time employees and will move headquarters to the U.S. during 2010

- Strong investor base
 - Sofinnova Partners (France), Gilde HealthCare (Netherlands), TechnoStart (Germany) and management



MANAGEMENT TEAM

Jan Møller Mikkelsen

Chief Executive Officer

Founder, President and CEO, LifeCycle Pharma
Co-Founder, President and CEO, Profound Pharma
President of Pharmaceutical Division, Maxygen
Vice President, Novo Nordisk A/S

Harald Rau

Chief Scientific Officer

CSO and Co-founder, Complex Biosystems
Head of Chemistry, Graffinity Pharmaceuticals

Torben Straight Nissen

Chief Operations Officer

President and SVP, Moksha8 Biologics
Managing Director and SVP, Maxygen

Lotte Sønderbjerg

Chief Administrative Officer

Sr. Director, HR, and Finance Director, LifeCycle Pharma
Sr. Director, Finance and HR, Acadia Pharmaceutical

Dirk Vetter

Managing Director, Heidelberg Site

CEO, co-founder and board member, Complex Biosystems
CEO and co-founder, Graffinity Pharmaceuticals

Grethe Rasmussen

VP, Product Development
and Alliance Management

Managing Director, Maxygen
Department Manager, Novo Nordisk

Michael Wolff Jensen

Chief Financial Officer

Executive VP and CFO, LifeCycle Pharma
Senior VP and CFO, Genmab

Michael Beckert

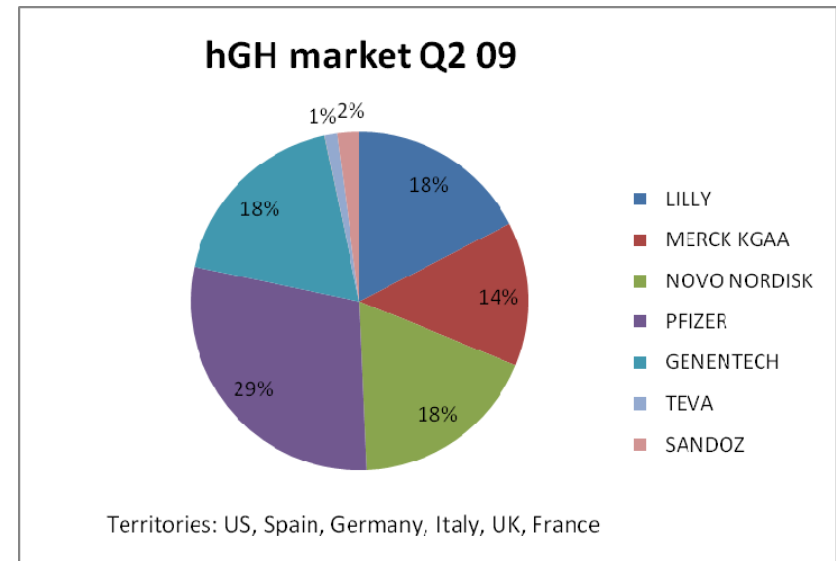
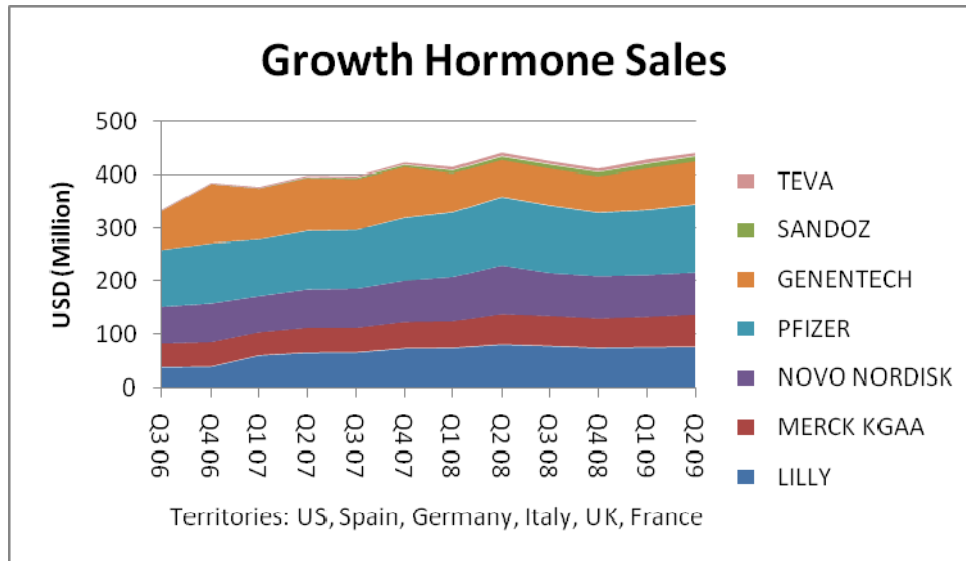
Chief Medical Officer

EVP and Chief Medical Officer, LifeCycle Pharma
Medical Director, SkyePharma

TransCon PEG Human Growth Hormone - *Improved convenience via once-weekly dosing*



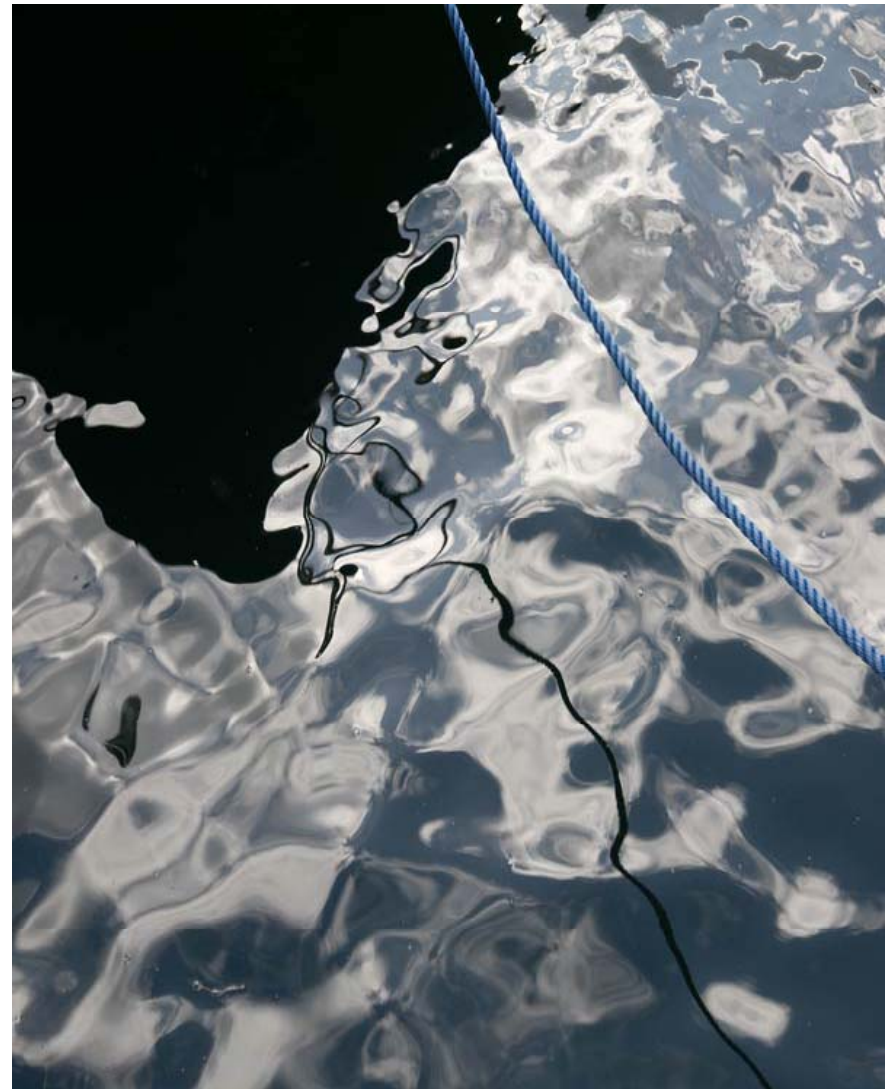
OVERVIEW OF THE HGH MARKET



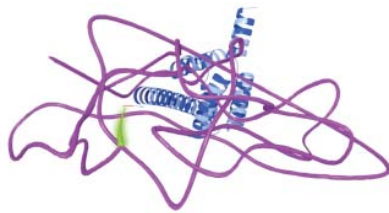
- Established specialty market with steady growth
 - High cost per patient
 - Long treatment period
 - Limited biosimilar penetration

RATIONALE FOR DEVELOPING TRANSCON PEG HGH

- Overcomes the inconvenience of daily injections
- Expand the adult growth hormone deficiency market through improved convenience and potential increase in metabolic efficacy
- Specialty market with a short and clear clinical pathway for regulatory approval
- Limited future competition as no products are emerging with gold-standard potential
- A once-weekly formulation with a new patent life allows Ascendis to maintain branded product prices, despite biosimilar competition



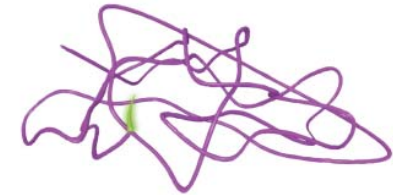
TRANSCON PEG hGH: ADVANTAGES



*Controlled cleavage of
TransCon Linker*



+



Inactive complex (Prodrug)

- No receptor activity
- Eliminates receptor clearance
- Eliminates renal clearance



Prevents injection site lipotrophy
Ensures a long duration of action

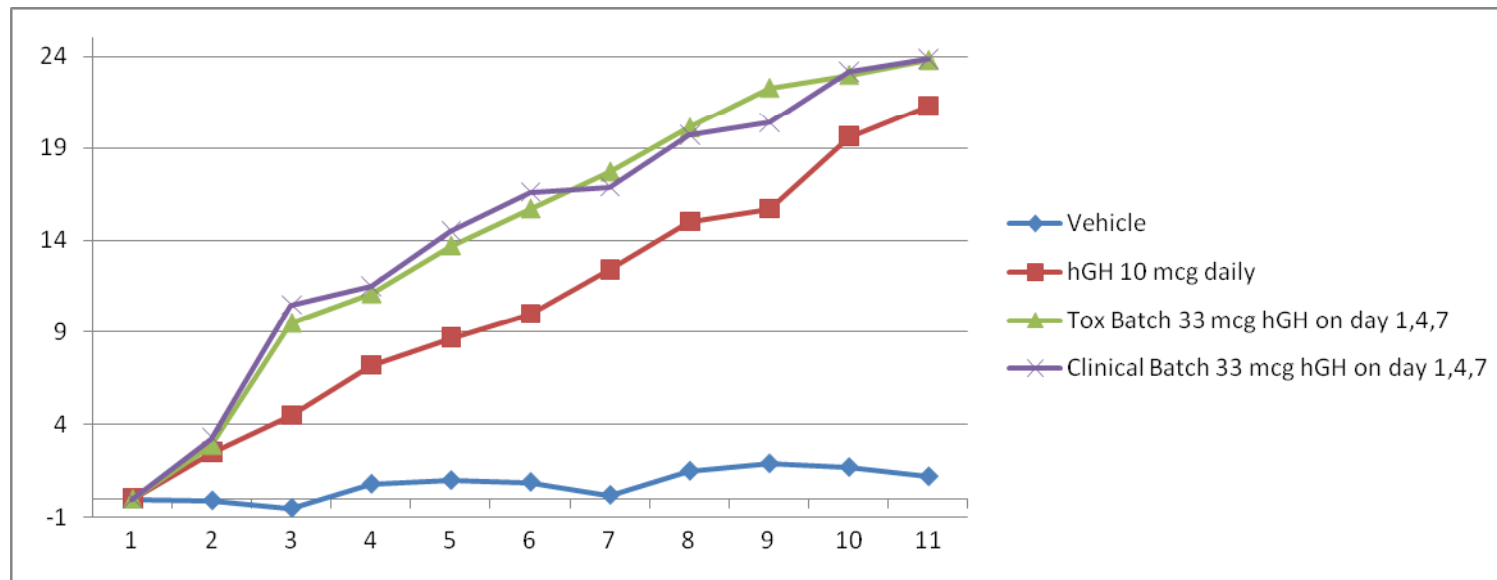
Native Growth Hormone

- Unmodified original drug
- Original bioactivity and distribution
- Predictable drug levels



Optimal longitudinal growth and
metabolic effects
Confirmed mechanism of action

HYPOPHYSECTOMIZED RATS WEIGHT GAIN



- Total hGH amount was identical in daily hGH and TransCon PEG hGH arms
 - Tox and Clinical Batch: 33µg hGH (167µg TC PEG hGH) on Day 1, 4 and 7
 - Daily hGH: 10µg hGH daily for 10 days

TransCon PEG hGH induced superior weight gain compared to equal amounts of daily administered hGH in hypophysectomized rats

OPTIMAL TARGET PRODUCT PROFILE

A group of experts were asked to describe an ideal TPP for a long acting hGH:
 Prescribers will take no compromise on efficacy & safety vs daily hGH

Efficacy:	Growth promoting efficacy must be at least equivalent to daily hGH injections IGF-1 levels should not be consistently greater than for daily injection (Induction of supra-physiological IGF-1 levels not acceptable)
Pain:	Local pain no greater than for daily injections
Frequency:	Ideal administration is once per week assuming efficacy as above
Injection Volume:	Below 0.75 ml and preferably below 0.50 ml (small children) Below 1 ml is acceptable for teenagers and adults
Flexibility:	Flexibility to address various needs (age, diagnosis, preferences)
Safety:	Must be comparable to daily administration Preferably to be administered with an easy to use device for self-injection

ACP-001 (TRANSCON PEG hGH) PHASE 1 (PK/PD) DATA

Well on track to deliver according to the feedback from experts:

Efficacy:



IGF1 response of ACP-001 at least comparable to corresponding dose of 7 daily GH injections (PI)

Pain:



Only mild and transient injection site reactions

Frequency:



PK Profile supporting once-weekly dosing

Volume:



Below 0.75 for children
Below 1 ml for Adults / teenagers.

Flexibility:



PGHD: Dose Range covers from 15 kg to 60 kg.

Safety:



ACP-001 was safe and well tolerated
No antibodies seen up to 42 days

SUMMARY OF VALUE PROPOSITION

- ACP-001 was safe and well-tolerated
- Only mild and transient injection site reactions
- Pharmacokinetic profile supporting once-weekly dosing
- IGF-1 response of ACP-001 higher than the corresponding dose of 7 daily hGH injections
- No test-article related adverse effects have been observed at a dose at least 15x higher than expected dose in humans and five repeated-dose over one-month
- Targeting EU and U.S. submission in 2013/2014



TransCon Hydrogel Insulin / Exendin -
The Future Gold Standard in Diabetes Treatment

EXECUTIVE SUMMARY

- Lead candidate for TransCon Hydrogel Insulin selected for once-weekly administration using autoinjector with 30 G needle
- Lead candidates for TransCon Hydrogel Exendin developed suitable for weekly to monthly dosing
- TransCon Hydrogel technology is optimal for combination therapy
- Future gold standards of type 2 diabetes treatment will be once weekly insulin, once weekly/monthly GLP-1 analog and fixed dose combination of insulin and exendin
- TransCon Hydrogel is a prodrug technology allowing 505(b)(2) filing with reference to selected approved API
- Scalable manufacturing process developed with commercially acceptable cost of goods sold (COGS)
 - Laboratory process has been developed to 1/10 of scale required for manufacturing of Phase I clinical material
 - Manufacture of hydrogel starting materials initiated

RATIONALE FOR THE DEVELOPMENT OF TRANSCON HYDROGEL INSULIN

- High treatment success rate, as reduced dosing frequency address non-compliance which affect at least 30% of type 2 diabetics
- Easy compliance as glucose control depends less on timing of dosing than current basal insulin
- Small intra patient variability allowing strict glucose control
- Type 2 diabetes treatment is moving towards weekly dosing with introduction of once weekly GLP-1 analogs
- The basal insulin market currently exceeds \$5 billion and is forecasted to exceed \$8 billion by 2014
- Prodrug technology allows for 505(b)(2) filing with reference to selected approved Insulin API

INSULIN MARKET

- The insulin market can be segmented into a long-acting insulin market (basal insulin) and a fast-acting market (prandial insulin)
- Only Sanofi-Aventis (Lantus) and Novo Nordisk (Levemir) have long-acting insulin (once-daily)
- Lantus is the fastest growing insulin product with a market share of 35% of the total insulin market and 2009 sales exceeding \$4.0 billion
- Levemir experiences sustained growth and reached blockbuster status in 2009

TRANSCON HYDROGEL INSULIN: COMPETITION

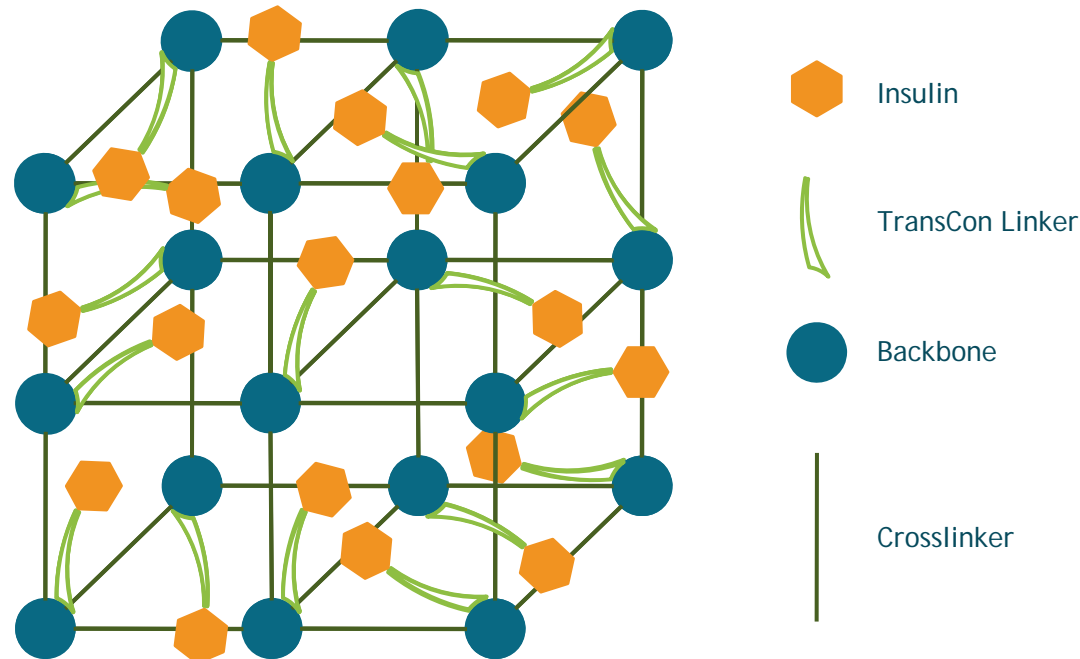
Basal insulins:

Overview of marketed products and clinical programs

Company Name	Drug Name	Dosing	Status
Sanofi-Aventis	Lantus	Daily	Marketed
Novo Nordisk	Levemir	Daily	Marketed
Novo Nordisk	Degludec	3 x week	PhII
Lilly	LY - unknown	Daily	PhI
Flamel	FT-105	Daily	PhI
Sanofi-Aventis	SAR161271	Daily	PhI
Lipoxen	SuliXen	Daily	PhI

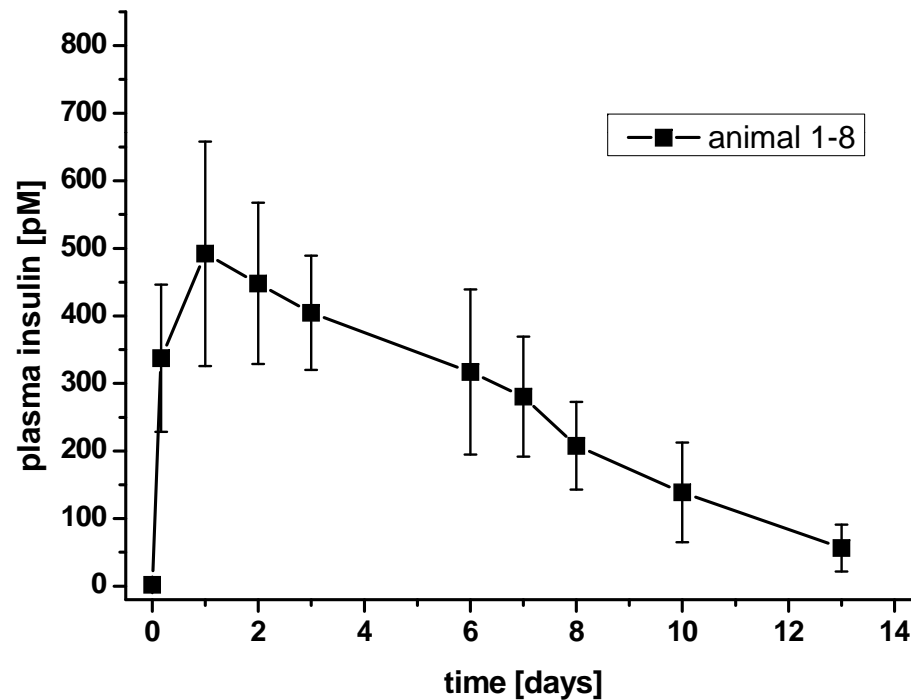
TRANSCON HYDROGEL PRODRUG TECHNOLOGY

- TransCon Hydrogel Insulin is built on Ascendis' proprietary TransCon Linker and Hydrogel technologies
 - After drug release the hydrogel collapse and PEG based components renally cleared
 - Compatible with pen system
 - Injectable through 30G needle



SINGLE DOSE PK STUDY IN RAT

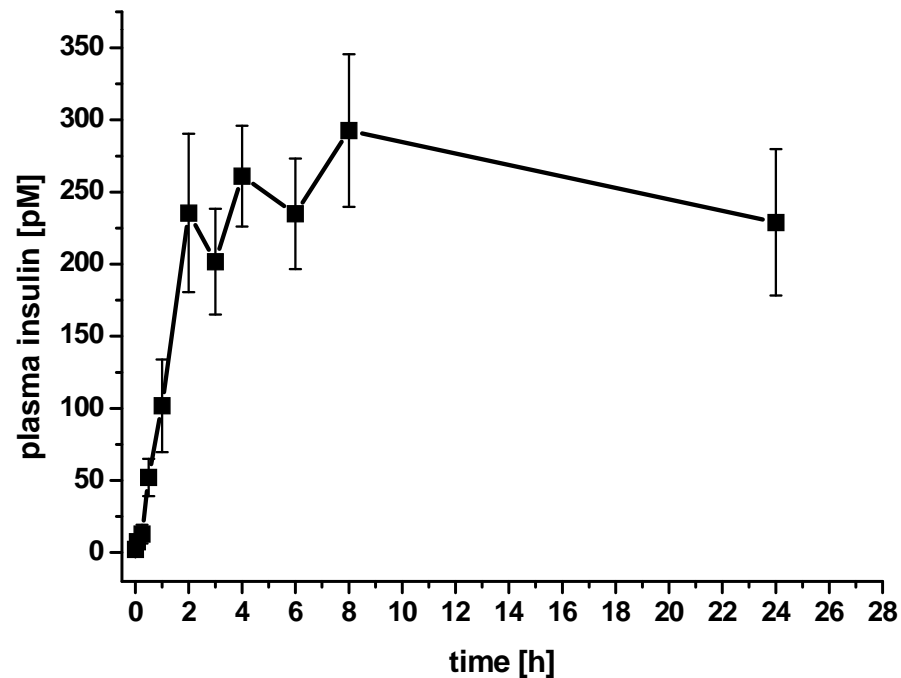
- Pharmacokinetics of TransCon Hydrogel Insulin after subcutaneous injection into rat (12 mg/kg dose; n = 8):



- Sustained release of insulin without any burst was observed

BURST ANALYSIS IN RAT

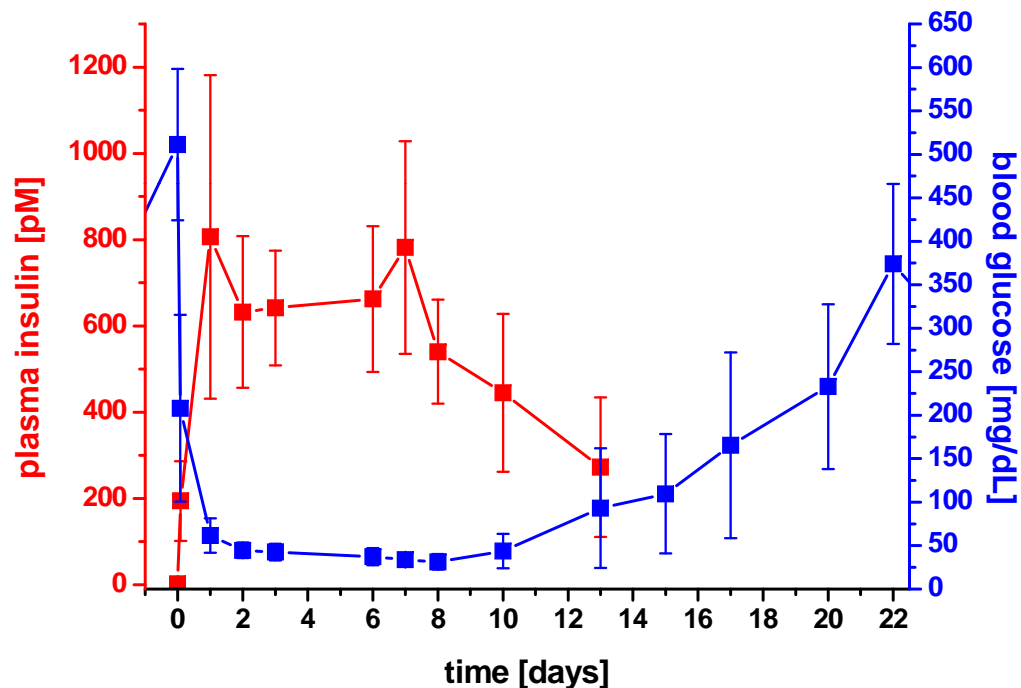
- Pharmacokinetics of TransCon Hydrogel Insulin (8 mg/kg) over 24 h after s.c. injection in rat. Two groups of four animals each were used (sampling time points group 1: t0, 5 min, 30 min, 2h, 4h, 8h; group 2: t0, 15 min, 60 min, 3 h, 6h, 24h).



- Insulin is released from TransCon Hydrogel Insulin without any burst.

SINGLE DOSE PK/PD STUDY IN STZ RAT

- Single dose PK/PD study of TransCon Hydrogel Insulin in STZ rats (24 mg/kg dose; s.c. injection; n = 8; PK in red; PD in blue)



- Sustained insulin release and blood glucose lowering over more than two weeks
- Good correlation between PK and PD
- Active insulin is released for more than two weeks

SUMMARY OF VALUE PROPOSITION

- Promising lead candidate for TransCon Hydrogel Insulin identified for once-weekly administration using auto injector with 30 G needle
- Scalable manufacturing process developed, with a commercially acceptable COGS
- TransCon Hydrogel Insulin optimally positioned for combination therapy
- TransCon Hydrogel Insulin will have a strong competitive edge in the basal insulin market, primarily due to its once-weekly administration



TransCon Hydrogel Exendin and Fixed Dose Combination

RATIONALE FOR DEVELOPING TRANSCON HYDROGEL EXENDIN

- TransCon Hydrogel Exendin offers predictable burst free PK profile with weekly to monthly dosing through a 30G needle in a small volume
- TransCon Hydrogel Exendin has the potential to be the only once monthly product
- TransCon Hydrogel Exendin can be combined with TransCon Hydrogel insulin in a fixed dose combination product
- The GLP-1 analog market represents a potential blockbuster market
- Prodrug technology allows for 505(b)(2) filing with reference to selected approved API

FIXED DOSE COMBINATION PRODUCT

- Fixed Dose Combination formulation of insulin and exendin for weekly administration developed allowing superior glucose lowering effects with less weight gain

- Identical hydrogel carrier is used for exendin and insulin
 - Tailor made release profile for exendin (30 days) and insulin (10 days)
 - No risk of deterioration of homogeneity of mixture
 - Injectability as either product alone using 30G needle in small volume



TransCon Hydrogel Paliperidone -
Superior long-acting antipsychotic depot

DEPOT ANTIPSYCHOTIC MARKET

- The antipsychotic market was \$18.2 billion in 2008, with injectable depots (Risperdal Consta) generating 2008 sales of \$1.3 billion
 - The injectable antipsychotic depot market is anticipated to reach \$2.9 billion in 2014
 - Invega Sustenna is priced at 9000 USD Vs. 4000 USD for oral Invega for yearly treatment*

- Increased awareness of beneficial effects of depot formulations in the US contributes to significant market growth of injectable depots

- Antipsychotics originally approved for schizophrenia are now also approved or undergoing approval in additional indications, including:
 - Acute schizophrenia, schizophrenia maintenance, schizoaffective disorder, acute bipolar, bipolar maintenance, bipolar depression, depression

- Introduction of antipsychotic depots offers optimal protection against generic erosion of the antipsychotic market value

* Medium dose regimens of paliperidone palmitate 117 mg every 4 weeks and paliperidone oral 6 mg/day

RATIONALE FOR DEVELOPING TRANSCON HYDROGEL PALIPERIDONE

- Subcutaneous administration of Paliperidone prodrug
 - Free choice of injection site
 - Small 30G needle is less invasive for the patient

- Therapeutic plasma levels reached within hours
 - No need for booster shot or oral supplements
 - Flat plasma profile for either one or two months
 - Proof of principle obtained in animal models

- Chemical coupling of Paliperidone to hydrogel carrier
 - Avoids formulation techniques with inherent high variability such as encapsulation and crystal formation
 - No risk of post injection sedation syndrome or initial burst release

TRANSCON HYDROGEL PALIPERIDONE: COMPETITION

Depot antipsychotics:

Overview of marketed products and development programs

All formulations require IM injection with 19-23G needle

Company Name	Drug Name	Dosing	Status	Issue
J&J	Risperdal Consta (Risperidone)	2 weeks	Marketed	Oral Rx
Lilly	Zypadhera (Olanzapine)	2-4 weeks	Marketed	Post injection syndrome
J&J	Invega Sustenna (Paliperidone)	4 weeks	Marketed	Booster shot on day 8
BMS	Abilify Depot Aripiprazole	4 weeks	Phase III	Potentially lower efficacy
Vanda	Iloperidone Depot (Iloperidone)	4 weeks	Phase I/II	n/a

OPTIMAL ADMINISTRATION

- TransCon Hydrogel Paliperidone is the only depot that can be administered subcutaneously through short 30G needle
- Administration of Invega Sustenna require either 1½ inch 22G or 1 inch 23G needle dependent on patient weight and administration site

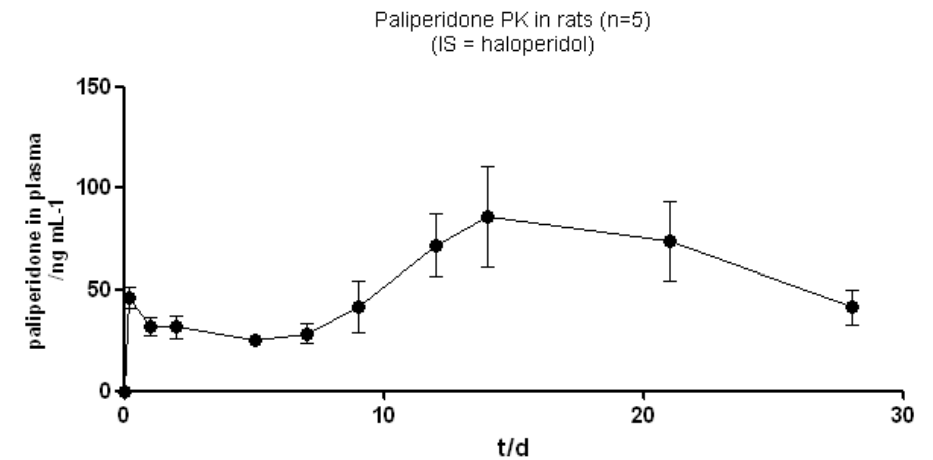


- Top: 22G needle used for IM administration of Invega Sustenna
- Bottom: Insulin type needle suitable for all s.c. administration of all TransCon formulations

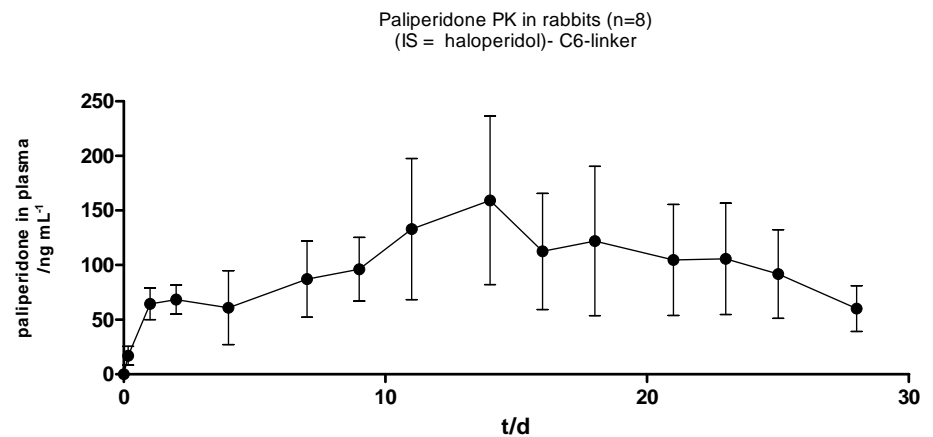


IN VIVO DATA IN RAT AND RABBIT

- Single dose study in either rat or rabbit demonstrates reproducible release kinetics
- In both species therapeutic plasma levels are reached within hours
- Flat plasma profile at efficacious concentrations for entire dosing period



Plasma profile of released paliperidone in rat



Plasma profile of released paliperidone in rabbit

SUMMARY OF VALUE PROPOSITION

- TransCon Hydrogel Paliperidone provides a superior treatment option compared to all other antipsychotics depot products
 - Convenient once-monthly subcutaneous injection with a 30 G needle
 - Immediate onset of action after injection without need for oral supplements or booster shots
 - No burst or risk of post injection syndrome

- Lead candidate for TransCon Hydrogel Paliperidone selected for once-monthly administration

- Scalable manufacturing process established

- Proof of principle obtained in animal models

- Specialty market with a short and clear clinical pathway for regulatory approval

- New Chemical Entity patents secure new product life span



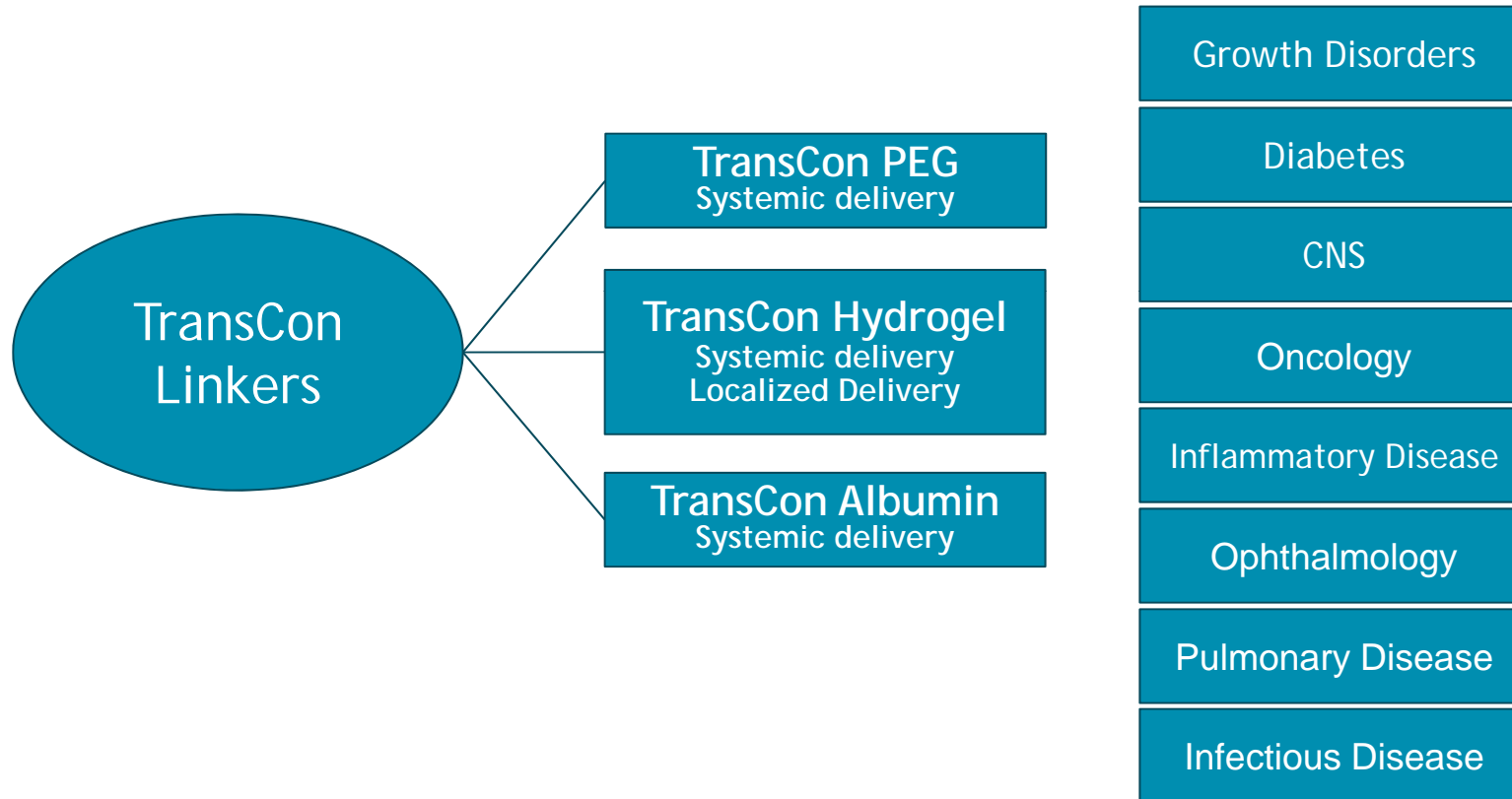
Pipeline & Technology

HIGH-VALUE PIPELINE

Therapeutic Area (Segment)	Product	TransCon Platform	Market Size*
Endocrinology (specialty)	Growth Hormone	PEG	\$3.0 billion in 2009
Endocrinology	Insulin	Hydrogel	\$>8.0 billion 2014
Endocrinology	Exendin	Hydrogel	\$0.7-1.5 billion in 2013
CNS (specialty)	Paliperidone	Hydrogel	\$2.9 billion in 2014
CNS (specialty)	Pramipexole	Hydrogel	\$3.4 billion in 2015
Infectious disease (specialty)	Interferon alpha	PEG	\$>3.5 billion 2014

* PROPAGATE PHARMA LTD

TRANSCON TECHNOLOGY APPLICATIONS



Combining TransCon Linkers with our various carrier systems creates a unique versatile technology platform, which can be applied in a variety of settings

TRANSCON LINKERS

- Self-cleaving linkers that autohydrolyze at a predictable rate
 - Linkers are stable under normal storage conditions
 - Release of drug initiated upon injection

- High precision tunable drug release
 - Drug release kinetics can be controlled to support daily, weekly, monthly or quarterly administration

- Predictable *in vivo* pharmacokinetic profile
 - Identical cleavage rates *in vitro* and *in vivo* enable predictable drug levels

- TransCon linker is eliminated with the carrier
 - No evidence of toxic degradation products

TRANSCON CARRIERS

	TransCon PEG	TransCon Hydrogel	TransCon Albumin
Carrier	Linear, branched, or 4-arm PEG	Proprietary, self-eliminating hydrogel	Albumin
Prodrug application	Proteins Peptides	Peptides Small molecules	Small molecules
Drug loading	1-4 molecules per PEG	50% w/w	1-6 molecules per albumin
Route and frequency of administration	Daily to fortnightly sc administration	Daily to quarterly sc administration	Weekly to monthly IV administration

TRANSCON PROVIDES CLINICAL BENEFITS

TransCon Technology		Clinical Benefit
Constant, controlled release of fully active drug	→	Increased efficacy
No burst effect Low peak to trough ratio	→	Reduced side effects
Administration of inactive prodrug	→	Reduced injection site reaction
Flexibility to select linker and carrier to allow daily through quarterly administration	→	Improved patient compliance

TECHNOLOGY ADVANTAGE

