Partnerships in Rare Diseases

To Partner or not to Partner?

IRDiRC Conference
Dublin, April 16, 2013

Hans GCP Schikan, CEO
Duchenne Muscular Dystrophy

Clinical symptoms:
- Walking problems
- Wheelchair - skeletal deformity
- Very limited use of arms
- Ventilation at night
- Ventilation 24 h
- Death
Duchenne Muscular Dystrophy

Cause: no dystrophin protein in muscles
‘Exon skipping’ can restore the reading frame
one medicine for more diseases

more medicines for one disease

personalized medicine
the biotech dilemma
to partner or not to partner
the biotech dilemma

to partner or not to partner
“There may be a total need for 4-5 computers in the world”

Thomas Watson
Founder IBM, 1943
“Radio has no future”
“Flying machines are impossible”

Lord Kelvin
President Royal Society, 1885
Maurits Cornelis Escher
1898 – 1972
Dutch graphic artist
PRESS RELEASE

Prosenza and GlaxoSmithKline form alliance to fight Duchenne Muscular Dystrophy

Leiden and London, October 13, 2009 – Prosenza, the Dutch based biopharmaceutical company focusing on RNA modulating therapeutics, and GlaxoSmithKline (LSE: GSK) announce that they have entered into an exclusive worldwide collaboration for the development and commercialization of RNA based therapeutics for Duchenne Muscular Dystrophy (DMD). DMD is a severely debilitating childhood neuromuscular disease that affects one in 3,500 newborn boys. Currently, there is no treatment to prevent the eventual fatal outcome.

The alliance was established under GSK’s Centre of Excellence for External Drug Discovery (ceedd) which seeks to collaborate with companies at the leading edge of highly innovative and transformative science. The scope of the alliance includes four RNA-based products intended to treat specific, but different, subpopulations of patients suffering from DMD.
trust
integrity
commitment
RARE DISEASES
Challenges and Opportunities for Social Entrepreneurs

Edited by Nicolas Sireau
living apart together
communication
communication or promotion?

newborn screening → enlarging ‘the market’
creating disease awareness → generating more revenues
informing patients → promotion to ‘consumers’
establishing registries → ‘lining up’ patients
scientific engagement
late or misdiagnosis
- Gaucher 74%
- Fabry 78%
- Pompe 67%

time lag from first visit to diagnosis
- Duchenne muscular dystrophy 2-3 yrs
- Gaucher 4-13 yrs
- Fabry 16 yrs
shaping the rare disease space is key

a different approach is needed
in comparison to common diseases

actively engaging in a small community
is mandatory
the rare disease community
patients as partners

talking **ABOUT** patients

talking **WITH** patients
communication with patients

- information
- investigational compound
- clinical trial
- treatment

- hope
- medicine
- treatment
- cure
decision making...
...can take a while...
small biotech
small biotech
Speed vs reach big pharma
Paying for the Orphan Drug System: break or bend? Is it time for a new Evaluation system for payers in Europe to take account of new rare disease treatments?

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<table>
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to partner or not to partner
Big Pharma vs Small Biotech

- development capabilities
- contacts with regulators
- payer relationships
- manufacturing know-how
- commercial infrastructure

- research capabilities
- patient proximity
- fast decision making
- entrepreneurial spirit
- sense of urgency
Ham. To be, or not to be, I there's the point, To Die, to sleepe, is that all? I all: No, to sleepe, to dreame, I mary there it goes, For in that dreame of death, when wee awake, And borne before an euerlasting Judge, From whence no passenger euer retur'nd, The undiscouered country, at whose sight The happy smile, and the accursed damn'd. But for this, the joyfull hope of this, Whol'd beare the scornes and flattery of the world, Scorned by the right rich, the rich cursed of the poore.
TO BE OR NOT TO BE
COME TO BECOME OR NOT TO BECOME A FULLY INTEGRATED COMPANY
the biotech dilemma

to partner or not to partner
yes, but...
Take the next left. No!! Go through the park... it's quicker.
partnerships
200 new therapies by 2020
Success Together

Together Success

Work Work Work

WorK WorK WorK