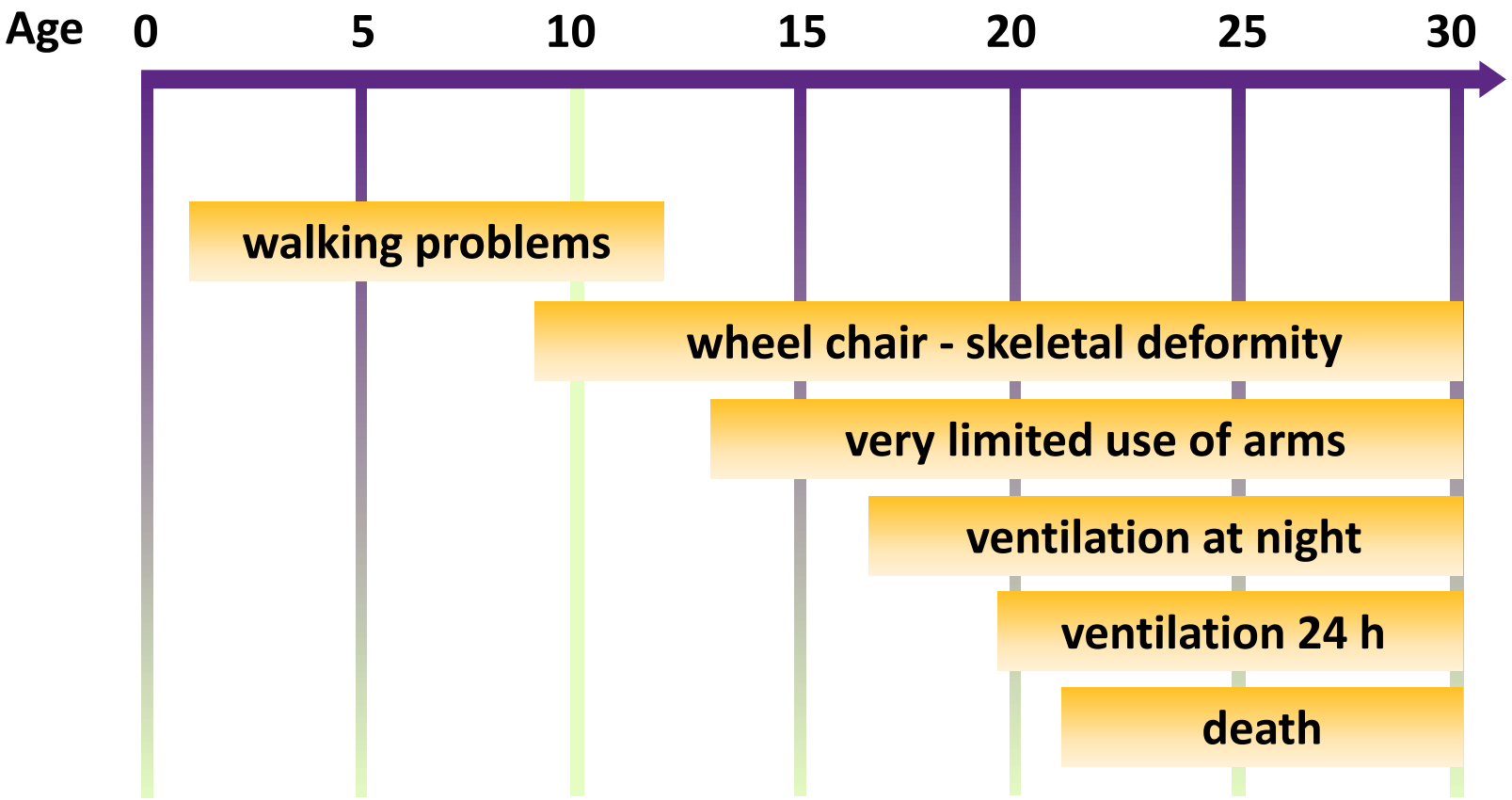
# Partnerships in Rare Diseases

# To Partner or not to Partner?

**IRDiRC Conference**
**Dublin, April 16, 2013**

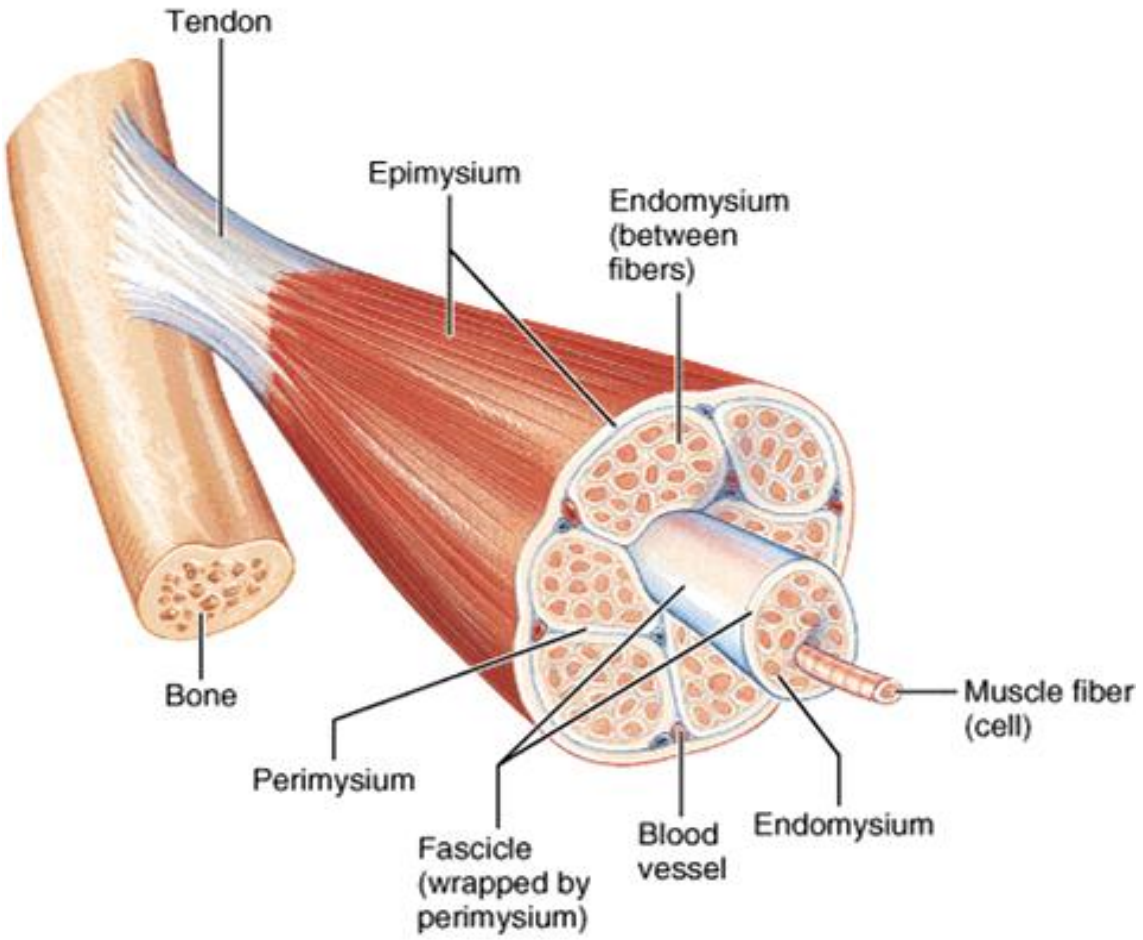
**Hans GCP Schikan, CEO**

# Duchenne Muscular Dystrophy

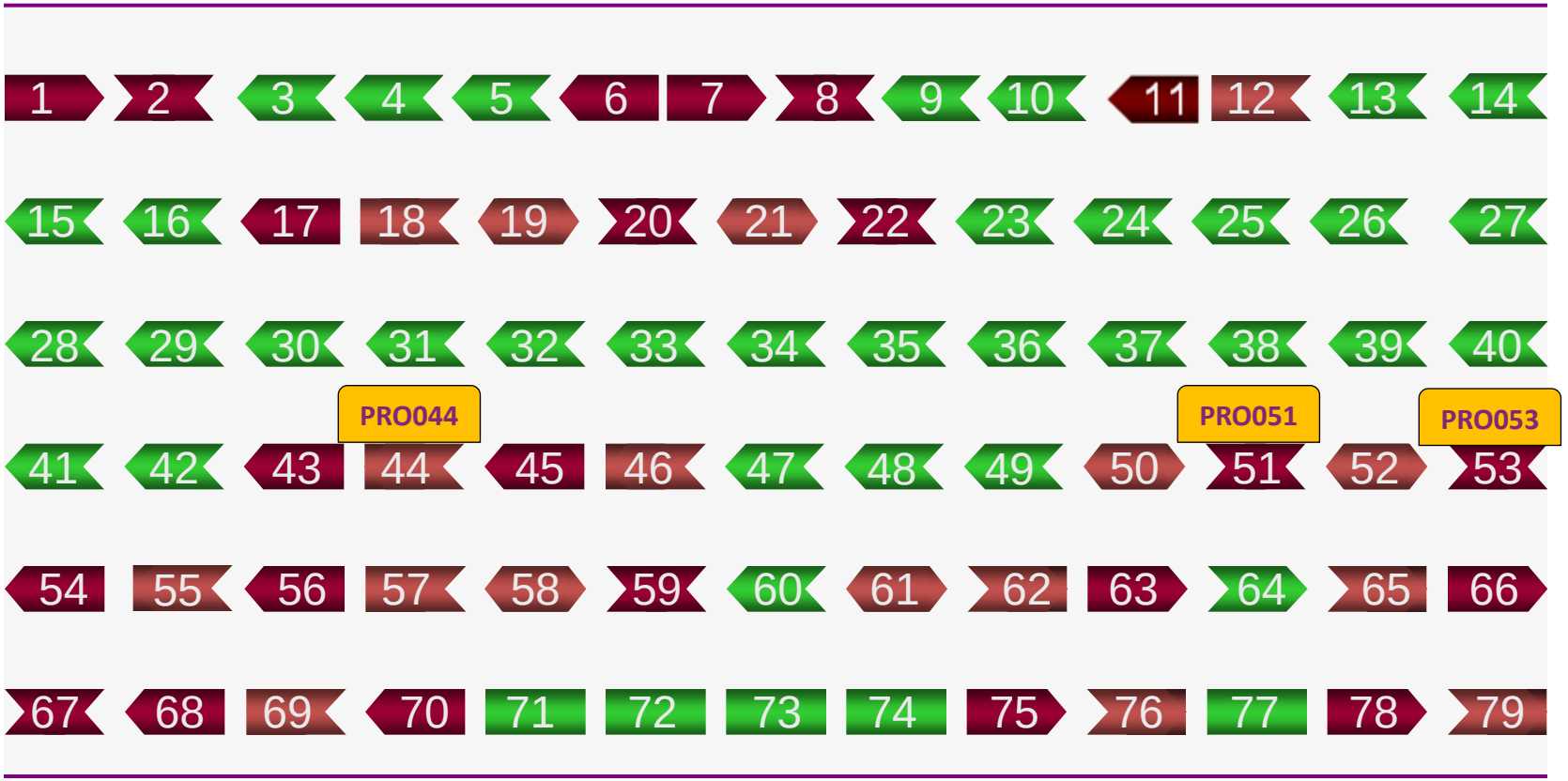


# Duchenne Muscular Dystrophy

**Cause: no dystrophin protein in muscles**

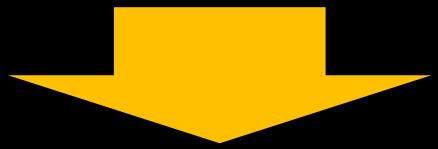


# The Dystrophin Gene




**‘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 bio[illegible]lemma

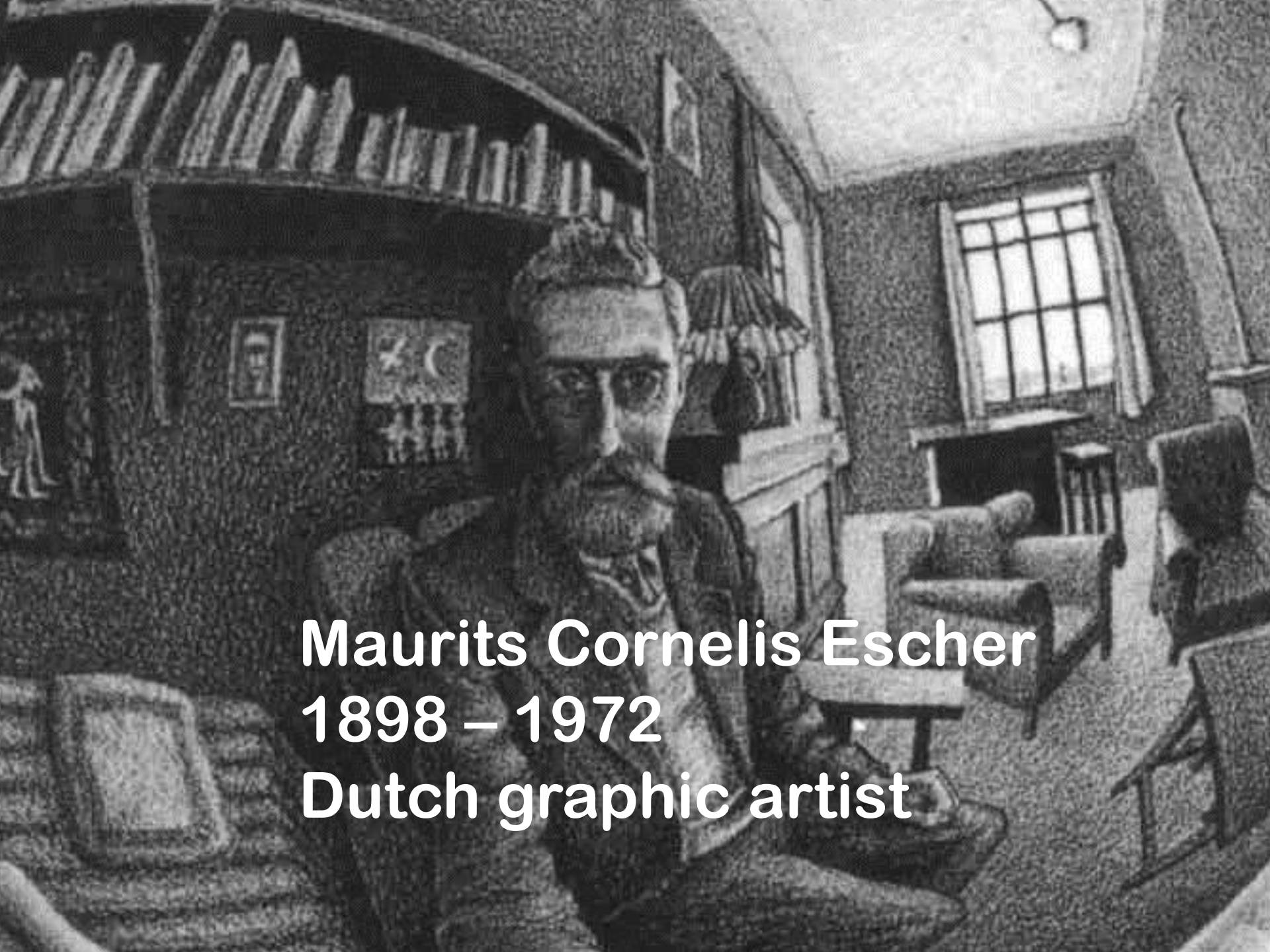
to pa[illegible]er or
not to partner

“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

2009

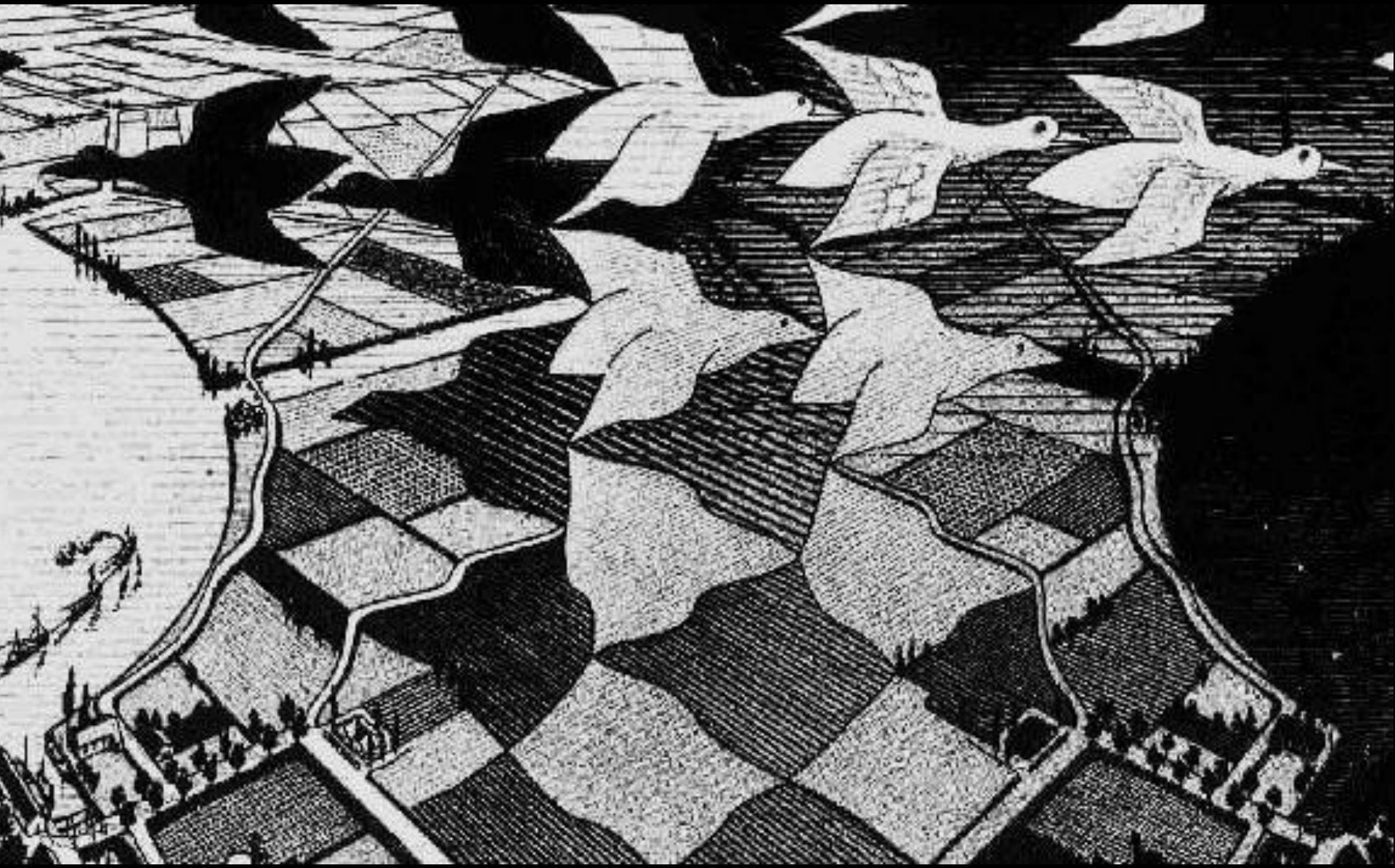
PRESS RELEASE

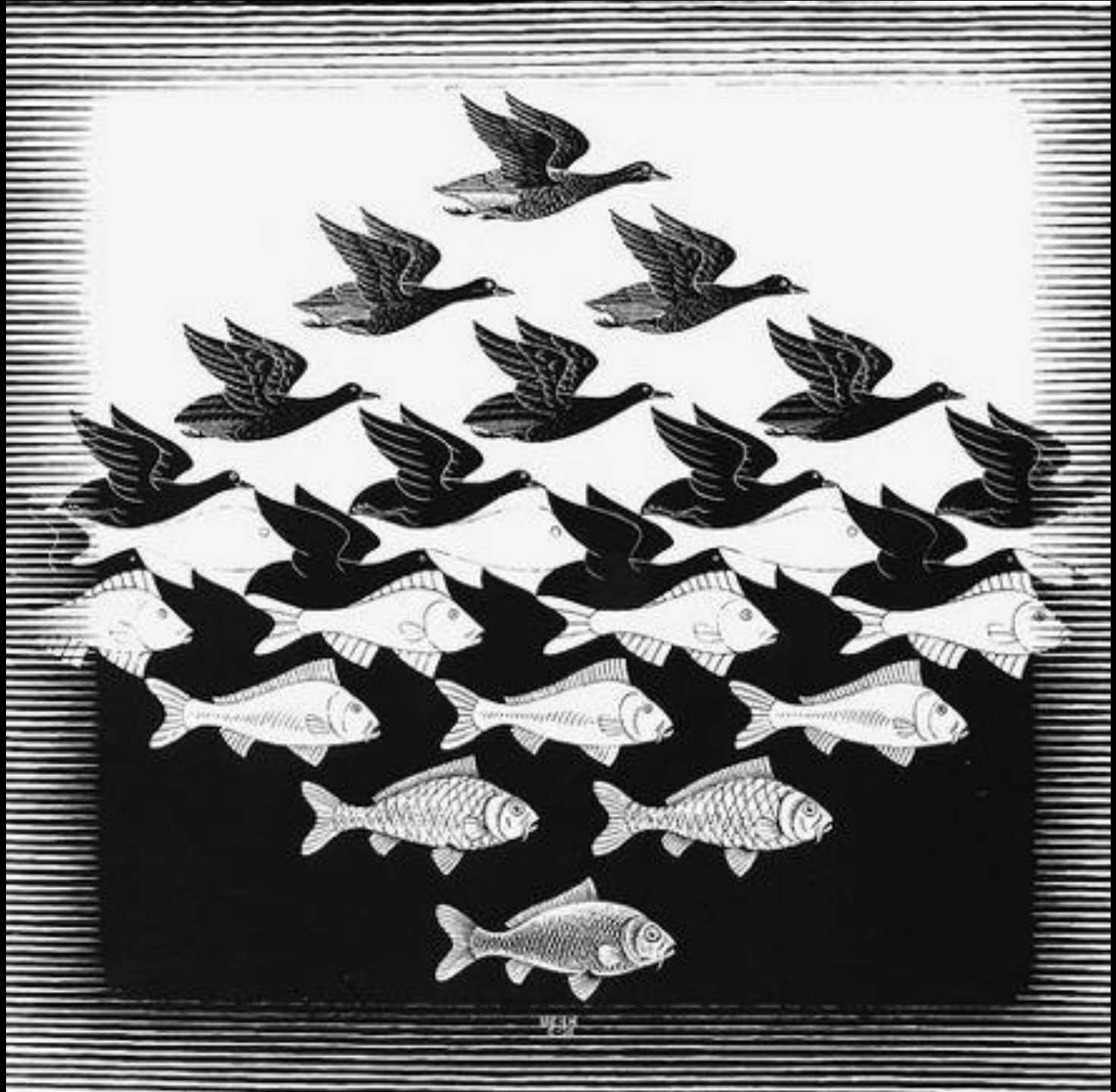
# Prosensa and GlaxoSmithKline form alliance to fight Duchenne Muscular Dystrophy

**Leiden and London, October 13, 2009 – Prosensa, 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.

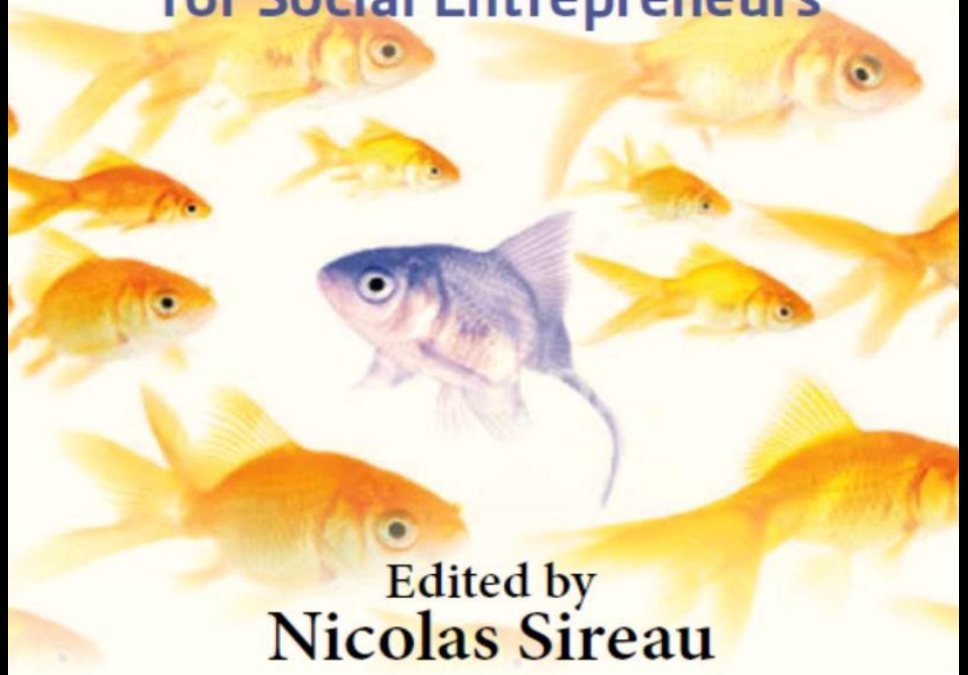
trust
integrity
commitment





# RARE DISEASES

## Challenges and Opportunities for Social Entrepreneurs



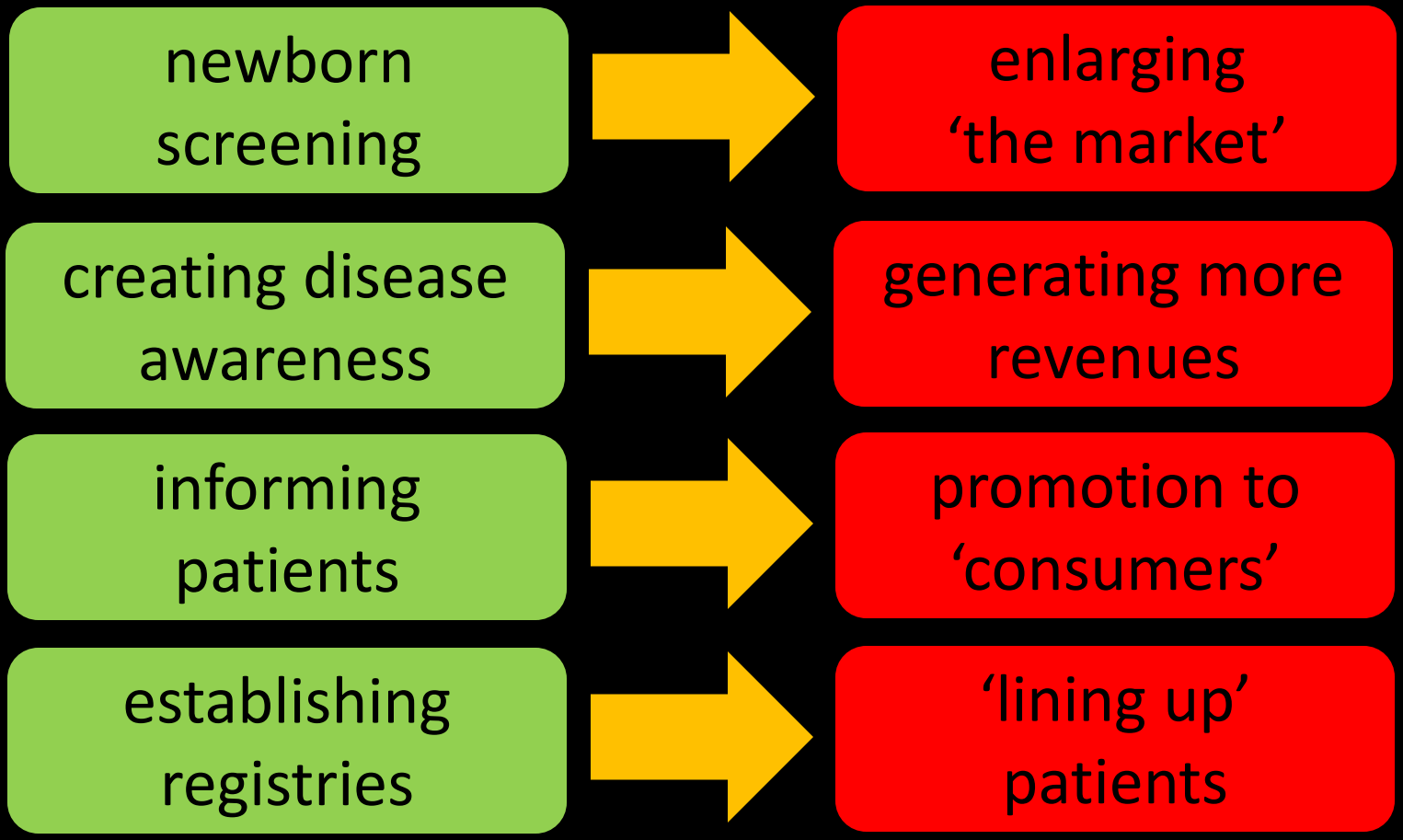
Edited by

Nicolas Sireau

# living apart together

communication

# communication or promotion?



fr
facebook
in
twitter
YouTube
digg

# scientific engagement

GLAXOSMITHKLINE PUBLIC STATEMENTS

## GSK and Marketing Practices

GlaxoSmithKline (GSK) is committed to ethical, responsible, principled and patient-centered promotional practices. Our interactions with healthcare professionals are intended to benefit patients and enhance the practice of medicine.

In order to be truly successful in that endeavour, our practices conform to high ethical, medical and scientific standards that are determined by law and regulation, promoted by industry associations and embraced by the company. All GSK marketing and promotion is based on valid scientific evidence, is consistent with national prescribing information documentation and complies with all applicable laws and regulations established at national levels.

Corporate responsibility, including ethical marketing practices, is an essential part of our business. Sound business practices require us to operate in a manner that is responsible to all stakeholders, including our shareholders and society at large. That responsibility, along with a commitment to integrity, runs through the entire organisation.

**GSK Follows Recognized Marketing Codes**

Health authorities regulate product approval, labelling, information provided to the patient and product claims. In some countries, health authorities also regulate promotional materials and the provision of samples.

Above and beyond the regulatory requirements of governments, GSK has in place mechanisms to govern its sales and marketing activities at a number of levels. For example, our Corporate Policy on Pharmaceutical Marketing and Promotion Activity applies to all employees, suppliers, contractors, and agents everywhere in the world.

GSK also abides by industry guidelines and codes established by national industry associations as well as the key guidelines from major international industry associations. These codes and guidelines supplement government regulation and provide guidance and self-discipline for ethical marketing practices through the industry's own high standards and ethical principles.

As a multinational company, GSK sells products in more than 150 countries. The company is structured into four main commercial regions – the United States, Europe, Emerging Markets & Asia Pacific, and Japan - reflecting the size of some markets while recognizing the similarities amongst others. We abide by laws and industry codes for each of the four regions and have also adopted our own marketing codes for each region:

- In the United States, we abide by the PhRMA Code on Interactions with Healthcare Professionals and the PhRMA Guiding Principles on Direct-to-Consumer Advertisements of Prescription Medicines. GSK also has in place a set of "Commercial Practices Policies" that were developed to provide clear rules on how GSK employees should conduct themselves in their job.
- In Europe, GSK observes individual country laws, regulations and industry codes, including the European Federation of Pharmaceutical Industries and Associations (EFPIA) Code of Practice and has its own GSK European Promotion of Medicines Code of Practice.

gsk GlaxoSmithKline

## late or misdiagnosis

- Gaucher 74%
- Fabry 78%
- Pompe 67%

## time lag from first visit to diagnosis

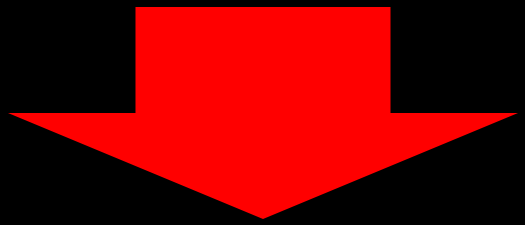
- Duchenne muscular dystrophy 2-3 yrs
- Gaucher 4-13 yrs
- Fabry 16 yrs

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

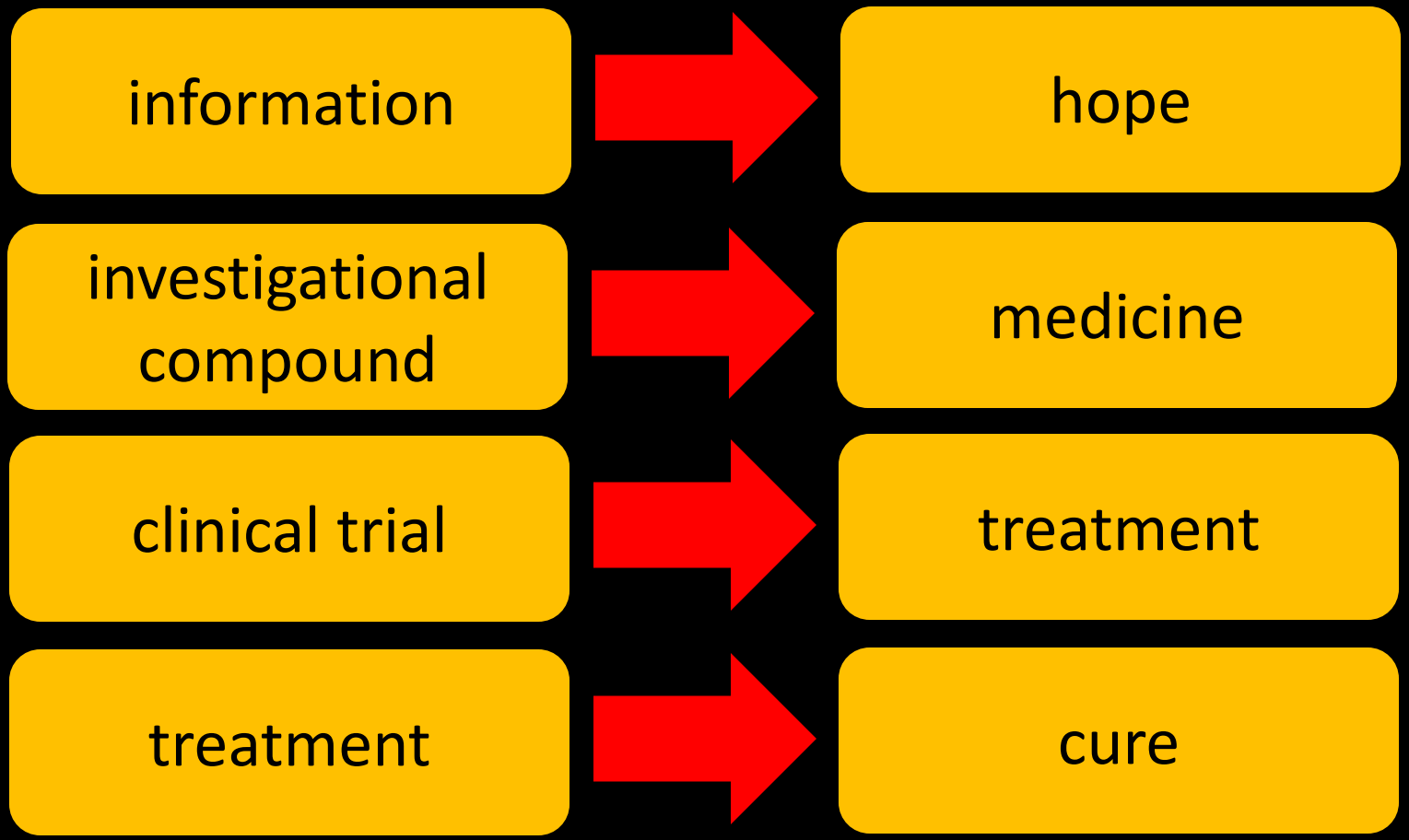
# patients as partners

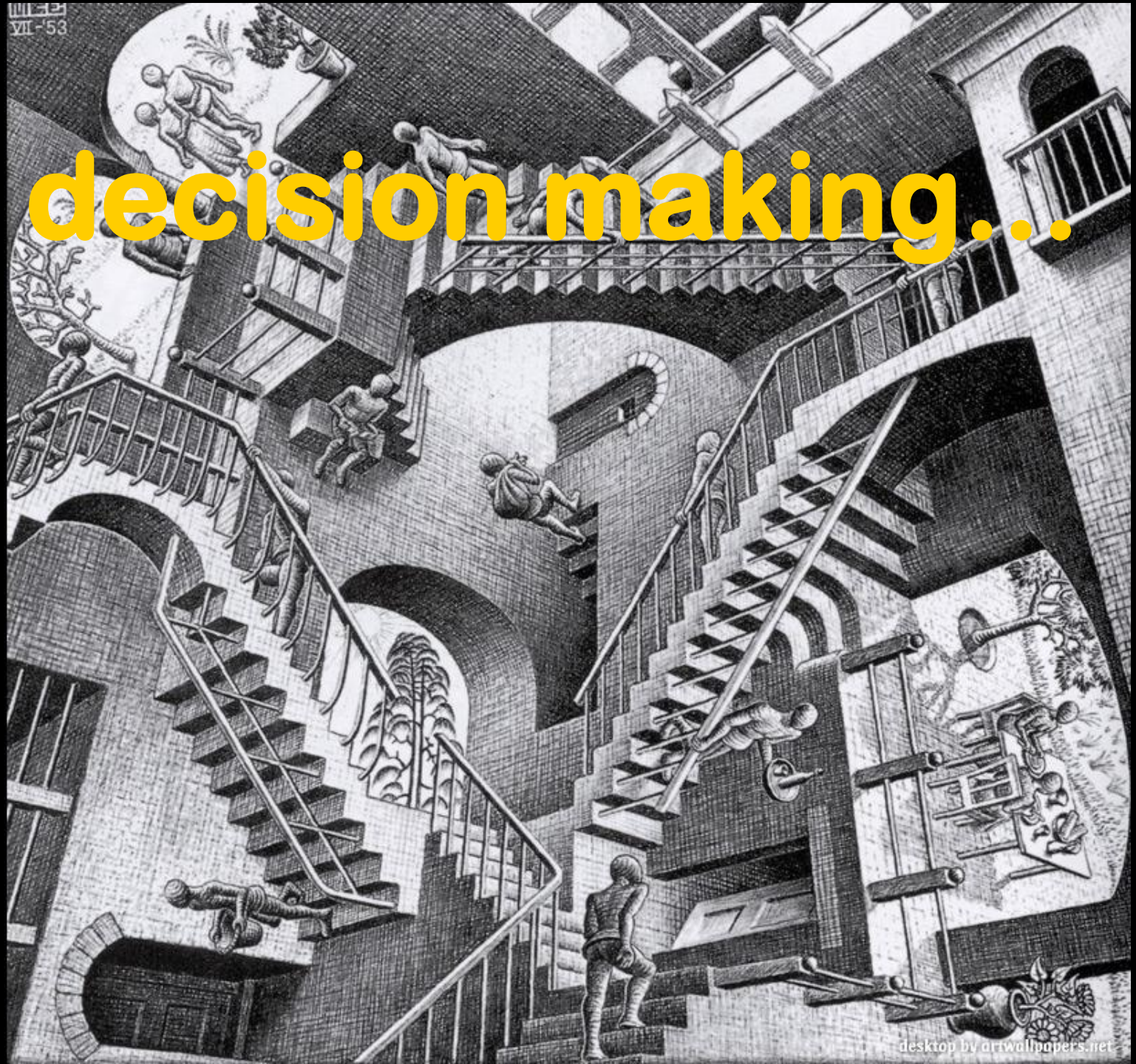
talking **ABOUT** patients



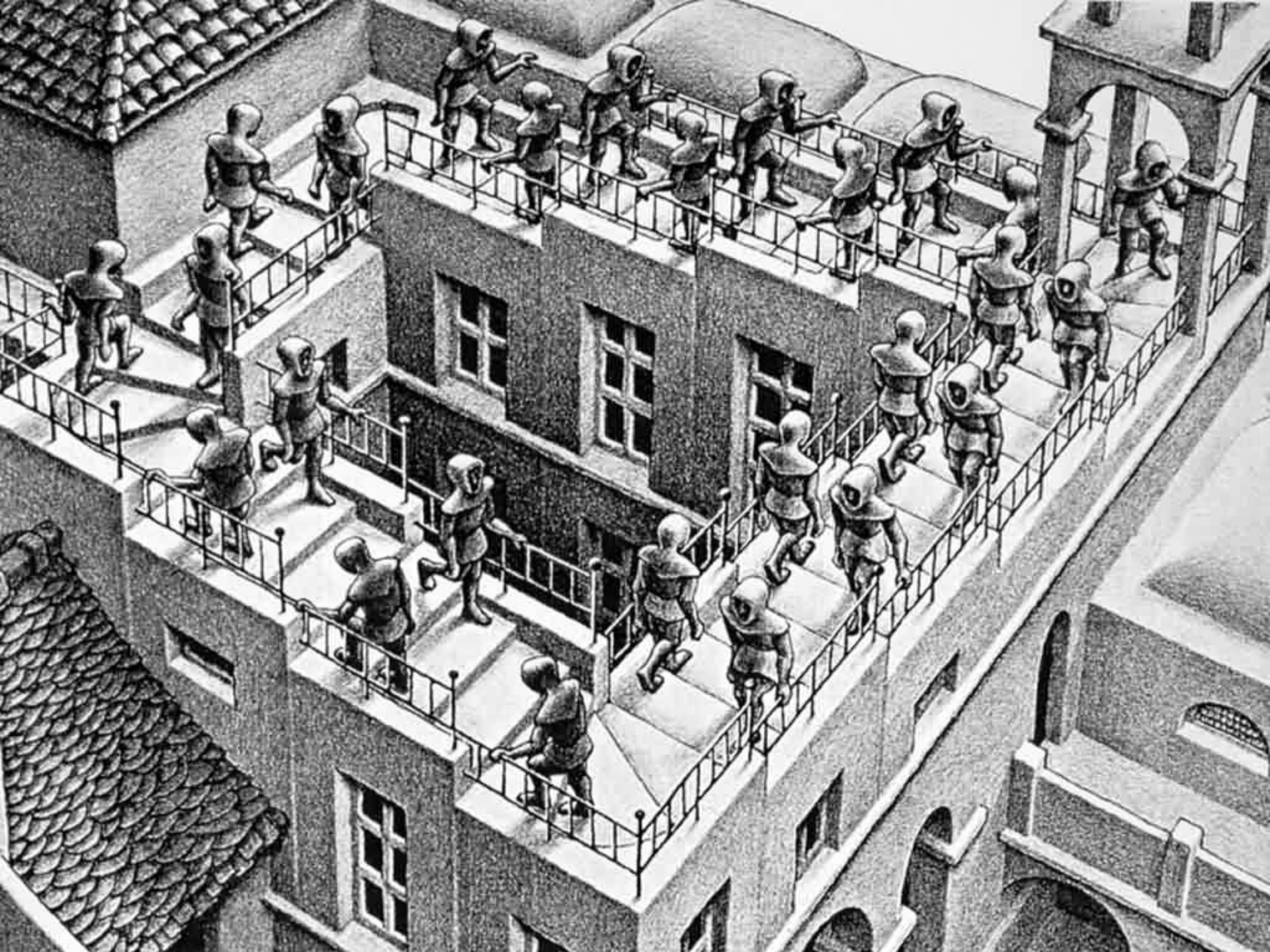
talking **WITH** patients

# communication with patients




decision making...
VII-'53
desktop by artwallpapers.net

…can take a while…



big pharma

small biotech

small biotech

big pharma

# sustainable pricing

# 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?

*Orphanet Journal of Rare Diseases* 2012, 7:74

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| proposed evaluation criteria |
| --- |
| rarity |
| investments |
| efficacy uncertainty |
| manufacturing complexity |
| follow-up measures |
| disease severity |
| alternatives |
| impact of treatment/disease modification |
| unique use |

to partner
or
not to partner

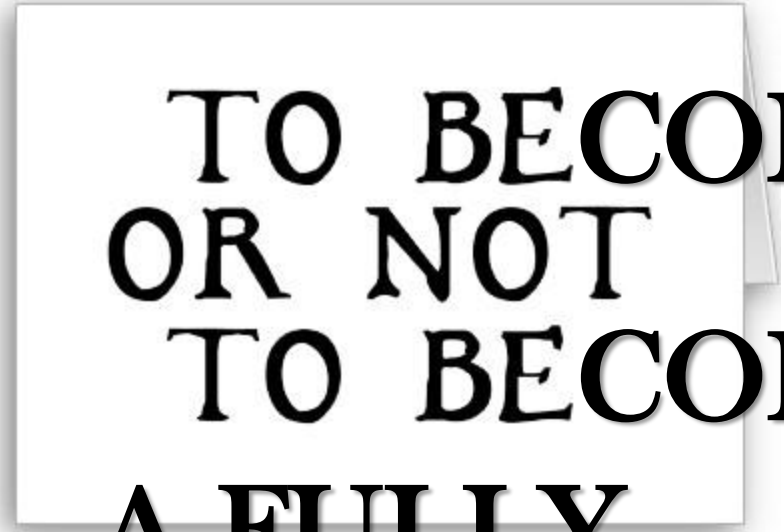
# Big Pharma vs Small Biotech

- 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 Iudge,
From whence no paſſenger euer retur'nd,
The vndiſcouered country, at whoſe ſight
The happy ſmile, and the accurſed damn'd.
But for this, the ioyfull hope of this,
Whol'd beare the ſcornes and flattery of the world,
Scorned by the right rich, the rich curſſed of the poore?

TO BE
OR NOT
TO BE




# A FULLY INTEGRATED COMPANY

# the biotech dilemma

## to partner or not to partner

**yes,** but…

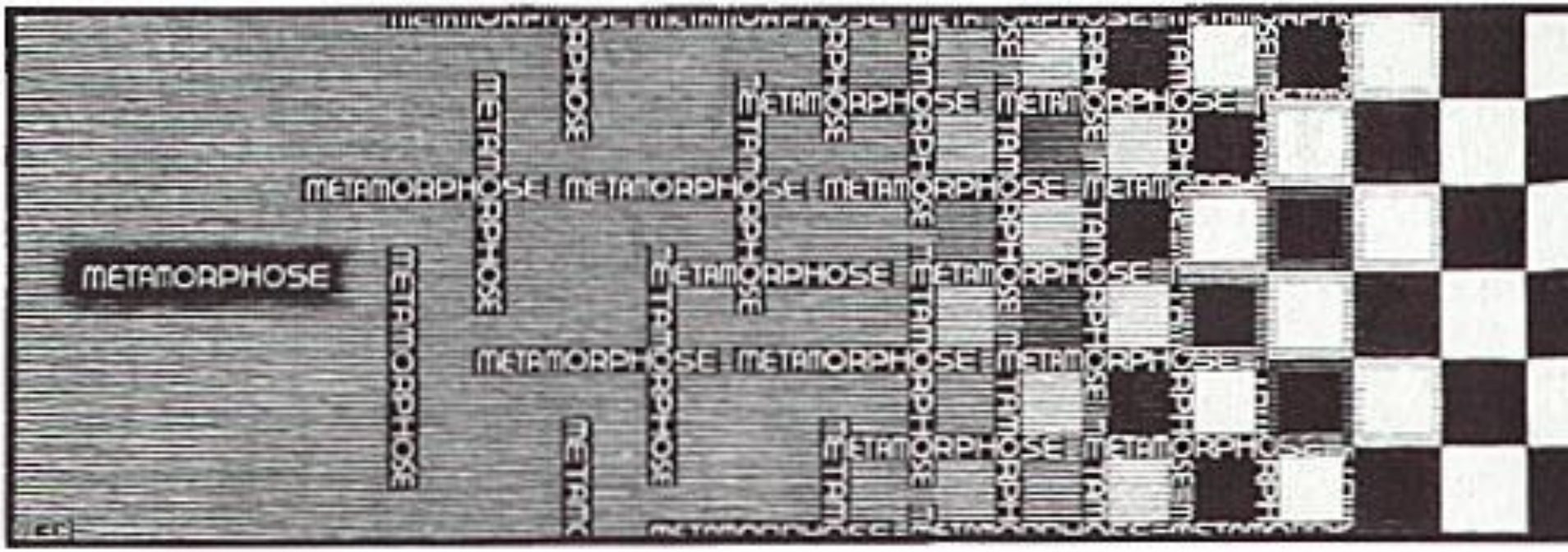
TAKE THE NEXT LEFT
NO!! GO THROUGH THE PARK... IT'S QUICKER
SATELLITE NAVIGATION SYSTEM
SATELLITE BACK SEAT DRIVER
RSJ

partner-
ships

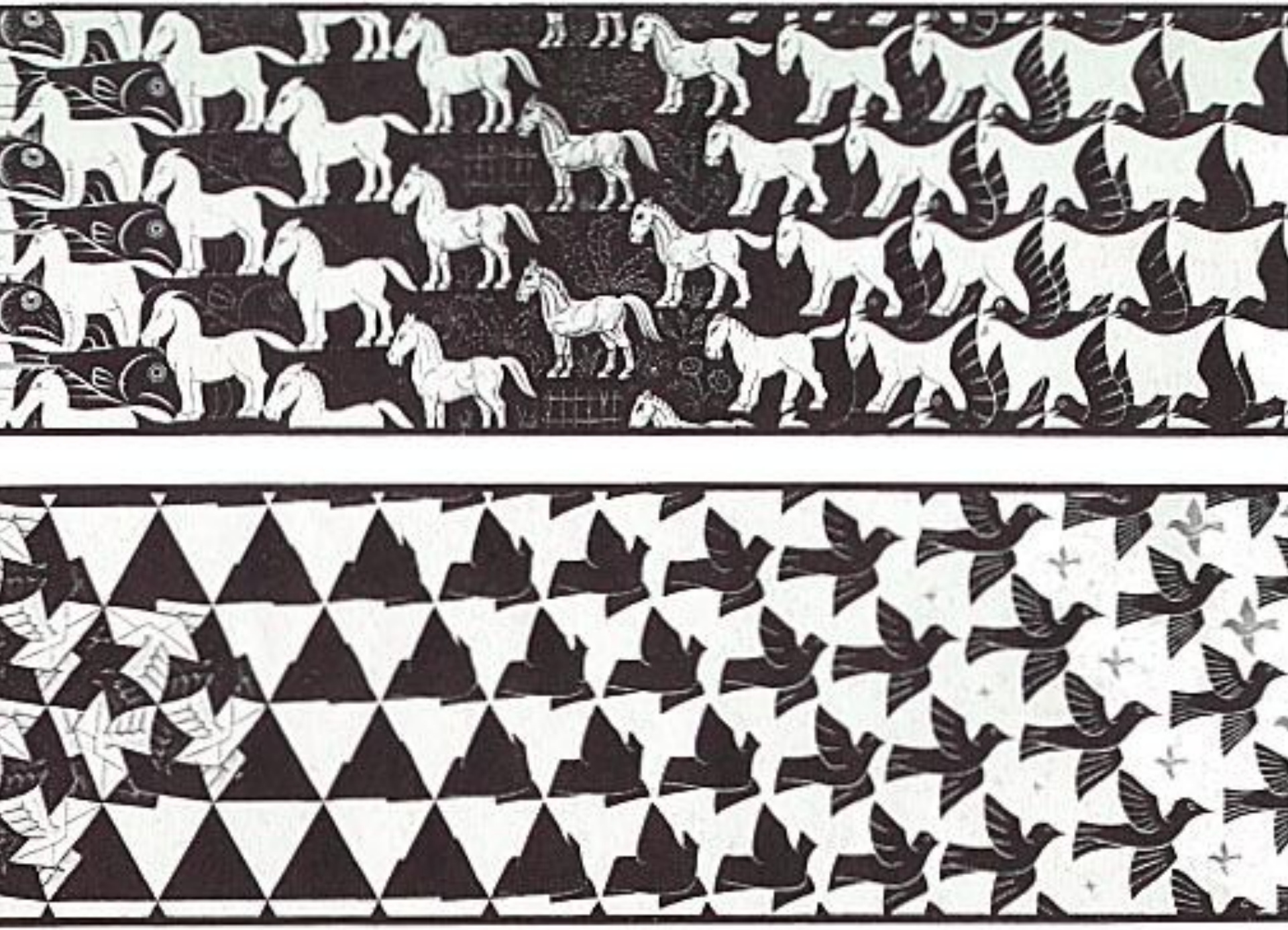
200 new therapies
by 2020

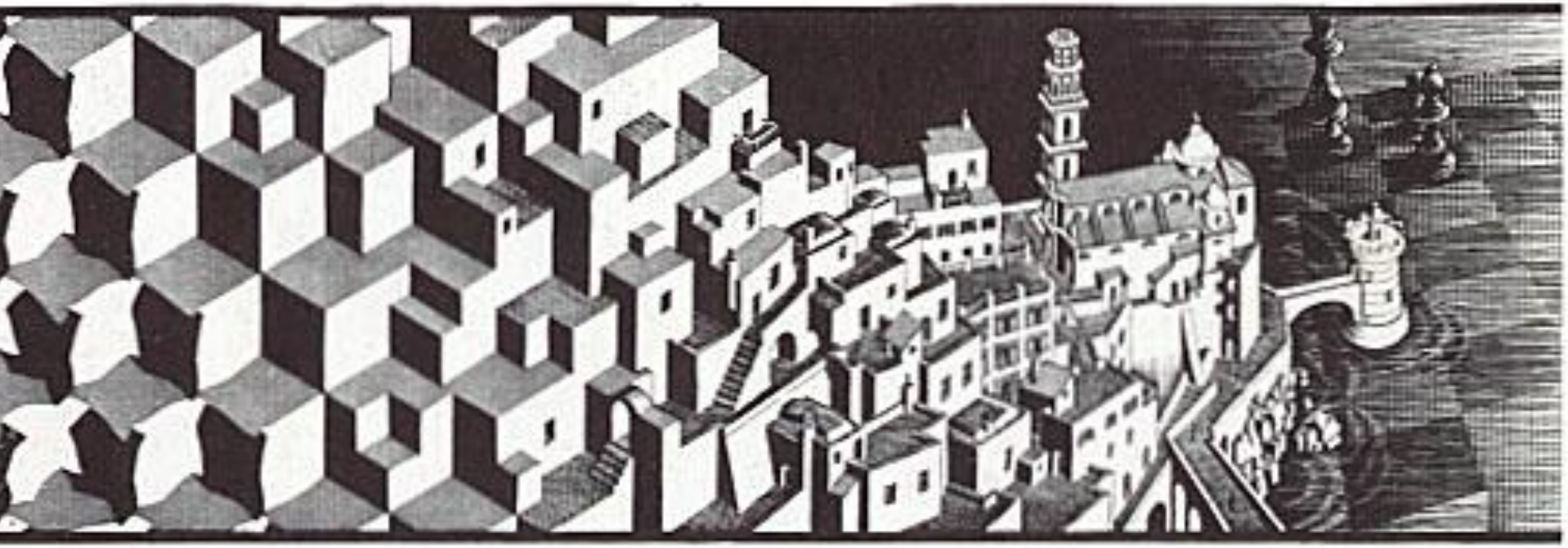
HYPAC

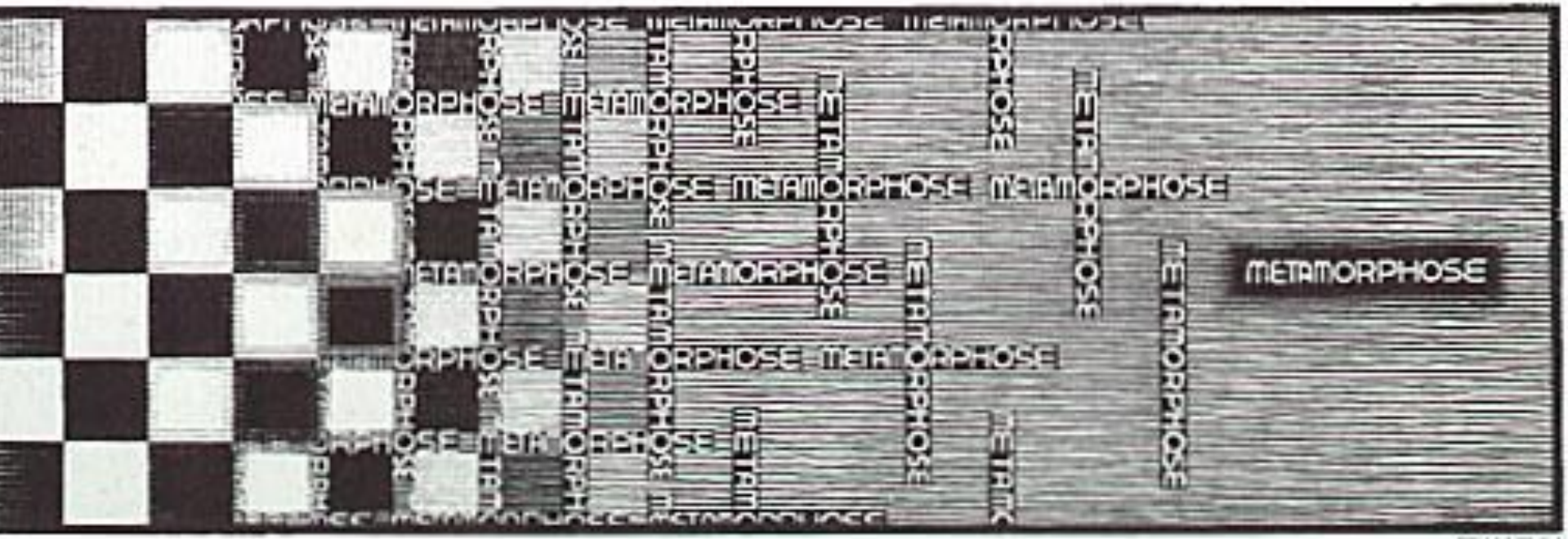
SUCCESS
WORK
GREETER


METAMORPHOSE

TWON





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