

HTAi – Adelaide 2023

Real World Evidence Generation for Rare Diseases.
A clinician perspective

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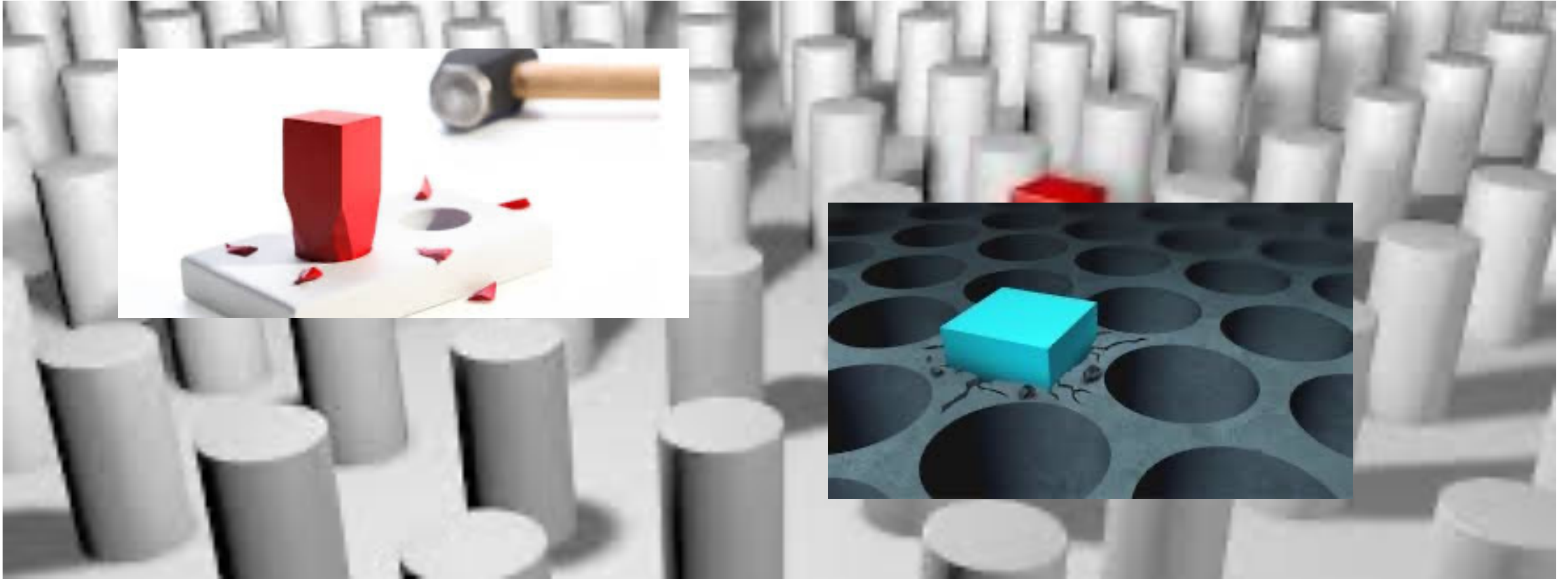
McMaster University

Disclosures, “personal” background, objectives

How can I, as a clinician, help bring [new/better] treatment options to MY patients with rare diseases?

- 1) Support patient co-design, in research and practice
- 2) Endorse the use of technology, in research and practice
- 3) Welcome innovative community thinking around data generation, ownership, ethics, and business models

The need for a paradigm shift



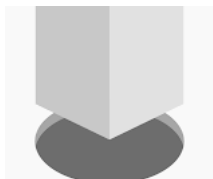
The need for a paradigm shift



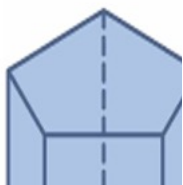
1. Industry
(including researchers, patent holder)
2. Regulators
3. HTA
4. Payers
(public vs private vs insurance based)
5. Patients
(including caregivers and HCPs)



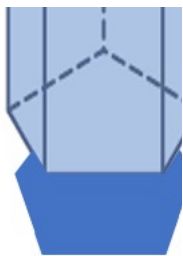
Patient with rare disease



"System"



Health for



"System"

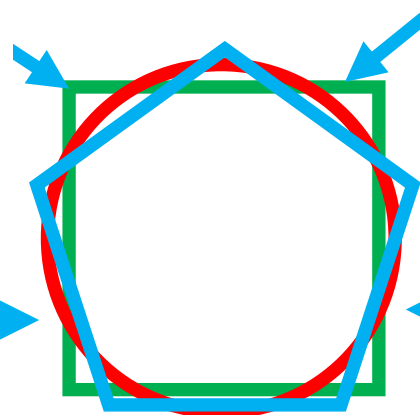
Industry (including researchers, patent holder)

- Risk sharing (including payment modalities, repayments when it does not work/stop working, etc)
- "health value" is what is monetized, not "cost of R&D"

Patients (including caregivers and HCPs)

- Pragmatic research will last for many years and enroll "most" patients as part of drug development
- This will have a "human cost" for many to pay to enable (very) future patients to enjoy the value.
- This (when accepted by everyone) will also greatly offset the cost of R&D.

HTA



Payers (public vs private vs insurance based)



- Uncertainty is factored in and may translate into
- a "discount"
- very specific guidance on which evidence to provide WITHIN THE BOUNDARIES OF THE DISEASE

- "Cost" for application and review is lowered depending on stage and size of the affected population (cost is covered charging more for larger markets)
- "Reviews" become "adaptive" and incorporates new evidence as it comes

Regulators

- "Health value" is what is monetized
- Innovative ways of assessing value are explored, e.g.
 - making values relative to each other
 - allowing harmonic and sustainable growth of the envelope

Co-design

Patients live and know the disease

Patients generate [own and can share] the data

- Pros

- Empowerment by sharing knowledge
- Learning together
- Building communities
- Evolve into advocacy

- Cons

- It requires huge commitment
 - Time, cost, psychological burden

- Risk

- Can be poisoned/hijacked
 - Looking for “one/that” solution, not looking for “value”

Technology

TECH / APPLE / SCIENCE

Apple's new health focus to elder care



ChatGPT Sprints to One Million Users

Time it took for selected online services to reach one million users



* one million backers ** one million nights booked *** one million downloads

Source: Company announcements via Business Insider/LinkedIn



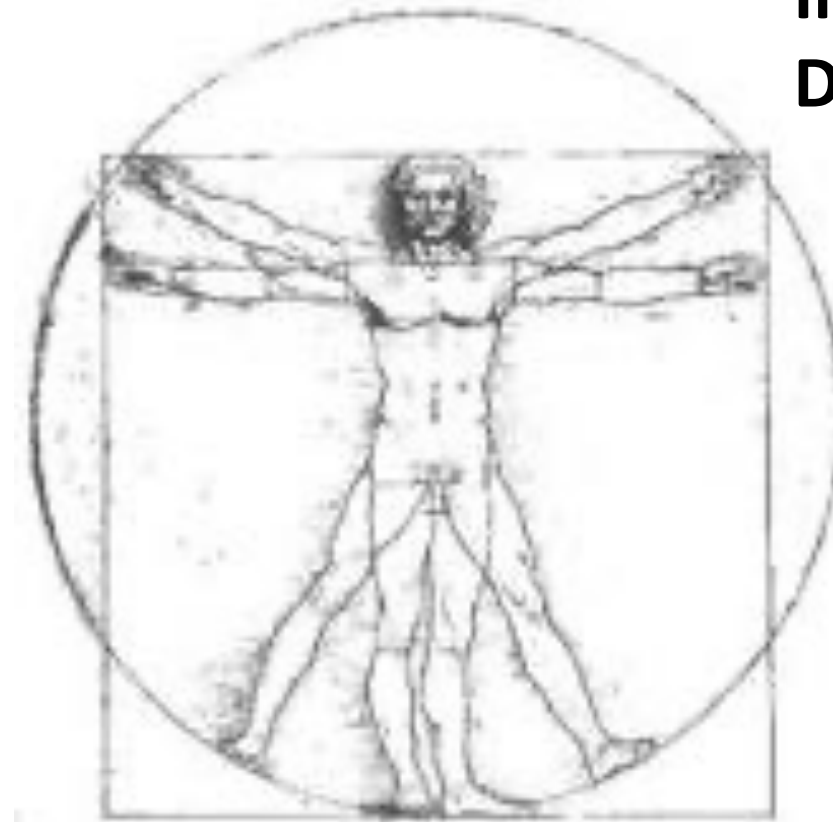
Welcome innovative community thinking around data generation, ownership, ethics, and business models



Take home message

- 1) co-design
- 2) technology
- 3) data generation,
ownership,
ethics, and
business models

Co-design



**Technology,
Innovation
Design thinking**

Power shift