HEALTH POLICY AND DRUG PRICING: A RESEARCHER'S PERSPECTIVE

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WHY THIS MATTERS TO ME

- I am a hematologist in Ottawa with 75% dedicated time for research
- I treat patients with hematologic malignancies (mostly leukemia, lymphoma, myeloma and bone marrow transplant)
- My patients can have a very poor prognosis and need better therapies
- It is my goal to bring novel therapies to the clinic so that patients have access to treatments that could ultimately improve their chances of cure/survival

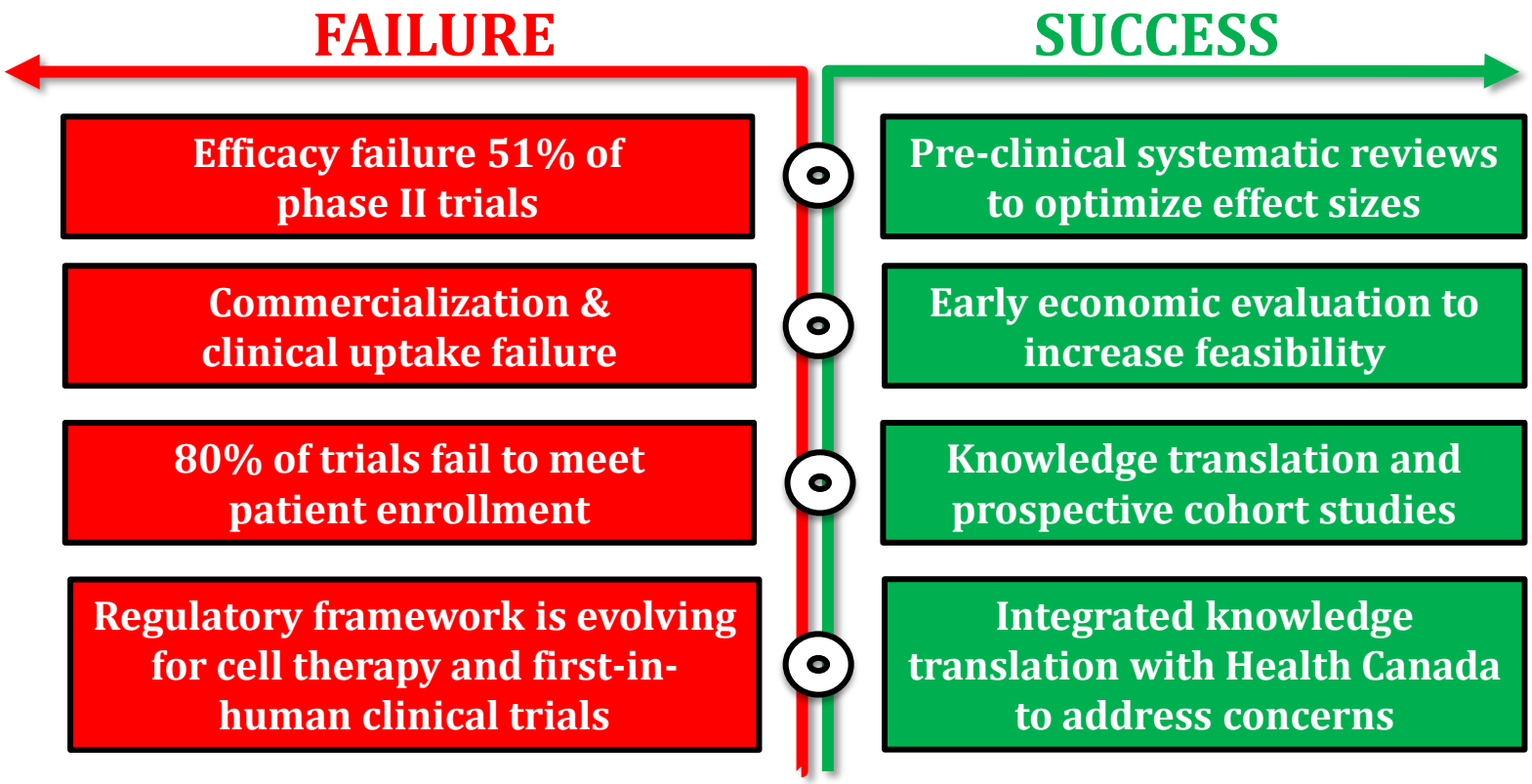
MY IDEAL WORLD

- Drugs or therapies that have good pre-clinical data should reach patients 90-100% of the time
- This pre-clinical data should translate into the clinic for practical use (NOT just research) within 3 years of the first-in-human trial
- Drug pricing, Health Canada approval, ethics approval, etc would not impede good pre-clinical data from reaching clinical trials
- Early phase clinical trials, when done appropriately, would be recognized by regulators such as Health Canada to bring forward novel therapies
- Pre-clinical and early phase clinical studies are well designed such that the treatment success rate is 90-100%

MY IDEAL WORLD

- Clinical trials would be collaborative, not competitive
  - Maybe more multi-center or national/international approaches
  - This improves access for patients to new therapies and makes results more generalizable
  - Especially important for patients with rare diseases
- Scientists and clinicians would always work together in bringing a new therapy to the clinic
- International trials would never be redundant but would complement each other, adding to the existing literature
- Promotions and salary or other rewards would never be the motivation to do research

HOW DO WE REACH THIS GOAL?



HOW DO WE REACH THIS GOAL? - STAKEHOLDERS

- Clinician Stakeholders
  - Interview clinicians from the centers involved in the study and ask to identify additional clinicians, including those with differing opinions (snowball sampling)
  - Use validated approaches to understand knowledge, beliefs about consequences, intentions, goals, social influences, and the emotion involved for clinicians
  - Involve multiple clinicians from multiple centers to improve the strength of the conclusions reached

HOW DO WE REACH THIS GOAL? - STAKEHOLDERS

- Patient Stakeholders
  - Should be involved LONG BEFORE a clinical trial is ever written!
  - Should participate in surveys that assess knowledge, beliefs about consequences, intentions, goals, social influences, and the emotion involved
  - Design and run prospective cohort studies as a “dry-run” with involved patients to determine patient interest, eligibility and numbers
  - This will help to assess barriers to implementing new therapies both for first-in-human trials and clinical practice

HOW PATIENT STAKEHOLDERS CAN ADD VALUE

- **Patients and patient support groups can advocate to government and regulators for the need of a new therapy**

- **Patient support groups can raise funding to initiate early phase clinical trials (that are often not pharma supported)**

- Patients can lobby physicians to be involved in novel therapeutics
  - "Doctor, I heard about this trial in Place X – are you able to get me referred?"

- Patients can provide intel to the media and regulators that demonstrates the current gap in available therapies for a specific disease (we have already seen the impact of a patient's voice in the news!)

HOW DO WE REACH THIS GOAL? - STAKEHOLDERS

- Funding Stakeholders
  - Failure to acknowledge economic considerations delays commercialization, reimbursement, and ultimately acts a barrier to clinical adoption
  - Insurance companies and those that fund health care (ex. OHIP, Medicare, etc) need to be surveyed early to understand whether a therapy can feasibly reach clinical practice
  - I would argue funders should be involved even at the early clinical trial stage to streamline therapy to everyday practice
  - Pharmaceutical companies generally don't get involved early (FDA/Health Canada approval, patent, profit, commercialization, etc.)

AN EXAMPLE

- **FAILURE:** New immunotherapy that uses a patient's own immune cells fails to improve patient outcomes due to manufacturing failures
- **WAY TO REACH SUCCESS:**
  - "Dry-run" to manufacture the product from patient samples without actually using it in patients to troubleshoot manufacturing issues
  - "Dry-run" prospective cohort study to determine if the eligibility criteria for patients should have been adjusted
  - Discussion amongst competing labs to identify errors and protocol glitches that may have led to decreased yield in manufacturing

HOW WOULD THESE CHANGES SHAPE OUR FUTURE?

- If clinicians, patients and funding agencies were involved early, we would be set up for **SUCCESS** in moving therapies from the lab bench to real-world patients

- This would mean not just clinicians designing and running trials, but **patients and funding agencies being involved in the protocol design**, ethics approval and implementation of the trial

- With early involvement, patients would have better treatment options as multiple new therapies would reach the clinic faster than ever before

THANK YOU

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