Stem Cell Network | Réseau de Cellules Souches

Incorporating ethical, legal, and social implications to strengthen stem cell science and knowledge mobilization

Case Study - ELSI Panel Discussion 2022

Till and McCulloch Meetings 2022

When: Oct. 4, 2022
12:15-2:20pm PDT
Where: English Bay Room on the 34th floor of the Hyatt Regency Vancouver

Part A: Basic Science Category

Panelists: Dr. Liisa Galea and Dr. Elizabeth Rideout

Consider this scenario:

The year is 2050. A recent paper described a new type of stem cell found in mice and humans that the authors termed 'SuperStems'. SuperStems are a very rare population of cells that persist into adulthood. They have pluripotent differentiation capacity, but they can alternate between differentiated and undifferentiated states. Researchers are excited that these SuperStems could unlock powerful regenerative mechanisms in humans, similar to what is seen in salamanders.

Your lab is interested in screening drug candidates that could be used to trigger the pluripotent state of SuperStems to initiate a regenerative response endogenously. The lab has successfully isolated SuperStems from mice, following methods described in the original paper where only male cells were used. Using the same protocols, a graduate student in your lab is able to derive SuperStems from a female mouse. Thus, you decide to include male and female SuperStems in your initial in vitro drug-screening experiment. You find two promising drug candidates that appear to trigger SuperStem pluripotency; one drug candidate seems to work better on female cells, while the other seems to work better on male cells.

Discussion Questions:

1. Is the observed phenomenon an example of a sex- or gender-related difference? What is the difference between sex and gender? How are sex and gender measured, and can these be considered binary variables?

2. You decide to perform additional experiments to test one or both of the promising drug candidates in a mouse model. Which drug(s) would you test, and would your experimental design include use of male or female mice? What are some possible study designs you could consider, taking into account scientific rigour while avoiding excess use of mice (in accordance with the Three Rs)?

3. What biological factors could account for differences between male and female SuperStems *in vitro* and *in vivo*? How might these factors play a role when considering potential clinical translation into humans?

4. What are possible implications or consequences of your experimental findings from i) a biological perspective; and ii) a therapeutic perspective?

Part B: Clinical Category

Panelists: Dr. Dean Fergusson and Dr. Manoj Lalu

Consider this scenario:

You are part of a research team that has genetically engineered a prospective cell therapy to treat cardiovascular disease. Following pre-clinical testing, your team is now planning two trials - a Phase 1 followed by a Phase 2 randomized trial (assuming Phase 1 is supportive). Your team is in the process of developing eligibility criteria (i.e., inclusion and exclusion criteria) so that you can begin recruiting patients.

As noted in the Tri-Canada Policy Statement regarding Fairness and Equity in Research Participation: "The principle of Justice holds that particular individuals, groups or communities should neither bear an unfair share of the direct burdens of participating in research, nor should they be unfairly excluded from the potential benefits of research participation. Inclusiveness in research and fair distribution of benefits and burdens should be important considerations for researchers, research ethics boards (REBs), research institutions and sponsors. Issues of fair and equitable treatment arise in deciding whether and how to include individuals, groups or communities in research, and the basis for the exclusion of some."

Discussion Questions:

1. What factors should be considered when determining who is eligible to be included or who should be excluded from a research study? How do you ensure that eligibility criteria are appropriate and fair?

2. How do you recruit patients to participate in a study? What actions could you take to ensure your recruitment strategy considers participation of individuals from underserviced or underrepresented groups?

3. How can your Phase 1 trial help inform eligibility criteria for your Phase 2 trial?

4. What are some ethical considerations regarding the choice of a control group(s) for your Phase 2 trial (assuming Phase 1 is supportive)?

Part C: Policy Category

Panelists: Prof. Timothy Caulfield and Prof. Amy Zarzeczny

Consider this scenario:

Scientists have created a novel stem cell therapy that has undergone a Phase 3 Clinical Trial, and all necessary testing leading up to such a trial, to demonstrate that the therapy is safe, efficacious, and of suitable quality. Following review, Health Canada has approved the novel therapy for distribution in Canada. However, market approval from Health Canada does not mean that the therapy will be publicly funded.

You are part of the team responsible for determining a policy for distribution of this novel stem cell therapy. Your team is divided on what they think would be the best policy. One side argues that the treatment should be entirely paid for by the patients so that access to the therapy is faster and does not depend on severity of illness and eligibility of the candidate, like organ donation. The other side of your team argues that the treatment should be entirely paid for by the government so that receipt of treatment is not determined by socioeconomic status. Both sides of your team believe that their option provides more equitable access.

Discussion Questions:

1. What barriers to access would a patient face with a publicly funded treatment versus a privately available treatment?

2. Do you think either of these policies would have consequences regarding patients seeking unproven stem cell therapies? Why or why not?

3. Which policy do you think is the most appropriate? If neither, what approach would you propose instead?