Stem Cell Research Oversight (SCRO)

Regulatory Services for Clinical Trials

ClinicalTrials.gov, FDA INDs and IDEs

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Clinical Trials Resource Center (CTRC)
Stem Cell Research Oversight Committee (SCRO)
The University of Texas Health Science Center at Houston
H. Human Stem Cell Research Oversight (SCRO) Committee
All research involving human embryonic stem cells (hESCs) or human induced pluripotent stem cells (hIPSCs) conducted at the university by its employees and/or involving use of its facilities or resources must be reviewed and approved by the Human Stem Cell Research Oversight (SCRO) Committee before it is initiated. Some research involving hESCs or hIPSCs may require additional approval by the AWC, IBC and/or CPHS. For more information on the application process, please contact the SCRO office at scro@uth.tmc.edu.
The **SOURCE** and **POTENCY** of the Stem Cell Line Determine Whether SCRO Review is Required

- **Totipotent**
  - Totipotent Stem Cells
  - Blastocyst
  - Human Embryonic Stem Cells (hESCs)
  - Human Induced Pluripotent Stem Cells (hIPSCs)

- **Pluripotent**
  - Multipotent Stem Cells
  - Unipotent Stem Cells
  - Specialized Cells

- **Multipotent**
  - Oligopotent Stem Cells
  - Stem Cells (hESCs)

- **Oligopotent**
  - Unipotent Stem Cells
  - Somatic Stem Cells, Progenitor Cells, or Multipotent Stem Cells (MSCs)

“Reprogramming”
The **SOURCE** and **POTENCY** of the Stem Cell Line Determine Whether SCRO Review is Required

- **Totipotent**
  - Totipotent Stem Cells
  - Blastocyst
  - Human Embryonic Stem Cells (hESCs)
  - Human Induced Pluripotent Stem Cells (hIPSCs)

- **Pluripotent**
  - Human Embryonic Stem Cells (hESCs)

- **Multipotent**
  - Multipotent Stem Cells
  - Oligopotent Stem Cells
  - Unipotent Stem Cells

- **Specialized Cells**

- **Adult Stem Cells**
  - Also known as Somatic Stem Cells, Progenitor Cells, or Multipotent Stem Cells (MSCs)
  - "Reprogramming"

Specialized Cells

Cells

Cells

Cells

Cells

Cells

Cells

Cells

Cells
The *SOURCE* and *POTENCY* of the Stem Cell Line Determine Whether SCRO Review is Required

- **Totipotent**
  - Blastocyst
  - Human Embryonic Stem Cells (hESCs)
  - Human Induced Pluripotent Stem Cells (hIPSCs)
  - Adult Stem Cells

- **Pluripotent**
  - Human Induced Pluripotent Stem Cells (hIPSCs)

- **Multipotent**
  - "Reprogramming"

- **Oligopotent**
  - Specialized Cells

- **Unipotent**
  - Specialized Cells

*Adult Stem Cells* (Also known as Somatic Stem Cells, Progenitor Cells, or Multipotent Stem Cells (MSCs))
The *SOURCE* and *POTENCY* of the Stem Cell Line Determine Whether SCRO Review is Required
<table>
<thead>
<tr>
<th>Stem Cell Type</th>
<th>Creation for Research Purposes</th>
<th>Research Use</th>
</tr>
</thead>
<tbody>
<tr>
<td>human embryonic stem cells (hESCs)</td>
<td>YES</td>
<td>YES</td>
</tr>
<tr>
<td>human induced pluripotent stem cells (hIPSCs)</td>
<td>No</td>
<td>YES</td>
</tr>
<tr>
<td>human totipotent stem cells</td>
<td>YES</td>
<td>YES</td>
</tr>
<tr>
<td>human gametes</td>
<td>YES</td>
<td>YES</td>
</tr>
<tr>
<td>human embryos</td>
<td>YES</td>
<td>YES</td>
</tr>
<tr>
<td>human adult stem cells (non-neural)</td>
<td>N/A</td>
<td>No</td>
</tr>
<tr>
<td>human neural stem cells, of any source</td>
<td>depends on source</td>
<td>YES</td>
</tr>
</tbody>
</table>
Stem Cell Research Oversight (SCRO) Website

https://inside.uth.edu/scro/

- SCRO Application
- Document “SCRO Policy and Procedures”
- Document “Guidelines for SCRO Review”
Stem Cell Research Oversight (SCRO) Contact Information

• **SCRO Office**
  – SCRO@uth.tmc.edu
  – (713) 500-3587

• **Elizabeth Gendel**
  – Elizabeth.M.Gendel@uth.tmc.edu
  – (713) 500-3587
Regulatory Services

- I review all IRB submissions that go to the full board to determine:
  - If a study must be registered at ClinicalTrials.gov
  - If a study of a drug, biological product, or device might need to receive FDA approval before it begins *(that is, if an IND or IDE is needed)*

- Either Clinical Trials Resource Center (CTRC) or CPHS will notify your study team if any of the above are required

- CTRC can assist with:
  - Registration and Results Entry at ClinicalTrials.gov
  - Preparation and Submission of Application to FDA for Approval to Study an Investigational Drug or Device *(that is, an IND or IDE application)*
What is ClinicalTrials.gov?

• Online database of clinical trials
  – Protocol info, results, recruitment status, and contact info
  – Free, public resource
  – Anyone can search the database
Once a Trial is Registered, it is Assigned an NCT #

NCT # stands for National Clinical Trial #

NCT000000000
Why Register and Report Results at ClinicalTrials.gov?

• It’s the law! enforceable by fines
• NIH funding can be terminated or withheld
• It can affect the ability to publish in a medical journals
  – ICMJE = International Committee of Medical Journal Editors
• An NCT # is required by CMS for claims for research-related procedures
• Required per UTHHeath’s HOOP 186
• Can serve as a recruiting tool
Which Studies are Required to Be Registered at ClinicalTrials.gov?

• Most basically,
  – Interventional studies with a health-related outcome
    • Interventional = Subjects assigned to an intervention as part of a study protocol (and not as part of routine medical care)
Who Registers the Study?

• If the study is initiated by a UTHealth PI,
  – then the UTHealth PI is responsible

• If the study is NOT initiated by a UTHealth PI,
  – then the industry sponsor or lead site usually registers
When to Register at ClinicalTrials.gov?

• Per FDAAA, the Final Rule, and the NIH policy, require registration no later than 21 days after enrollment of the first participant

• BUT, ICMJE journals require registration at or before the time of first patient enrollment

THE BOTTOM LINE:
It’s best to register after IRB approval but before enrollment begins.
How to Register at ClinicalTrials.gov?

• Contact CTRC to get an Account
  – CTRC, clinicaltrials@uth.tmc.edu
  – Elizabeth Gendel, 713-500-3587, Elizabeth.M.Gendel@uth.tmc.edu
  – Shwetha Pazhoor, 713-500-3578, Shwetha.Pazhoor@uth.tmc.edu

• Contact Shwetha Pazhoor for Help With Registration

• NCT # typically available 2–5 business days after submission
Maintenance of ClinicalTrials.gov Record

• Record must be updated
  – at least once per year
  – 30 days after any of the changes detailed on:
Results Entry

• Results are required for clinical trials that are:
  – Required by law to register (some trials of drugs or devices)
  – NIH-funded (all clinical trials, even behavioral studies)
Results Entry at ClinicalTrials.gov

• Work with Elizabeth Gendel
  – 713-500-3587, Elizabeth.M.Gendel@uth.tmc.edu

• Results are due 1 year after the trial’s “Primary Completion Date”
  – “The date that the final subject was examined or received an intervention for the purposes of final collection of data for the primary outcome, whether the clinical trial concluded according to the pre-specified protocol or was terminated.”
Results Entry at ClinicalTrials.gov

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• Results are due 1 year after the trial’s “Primary Completion Date”
  – “The date that the final subject was examined or received an intervention for the purposes of final collection of data for the primary outcome, whether the clinical trial concluded according to the pre-specified protocol or was terminated.”
To Ensure Results are Entered on Time:

As soon as you see the last patient to collect the last piece of primary outcome data:

• Update “STUDY STATUS” section in the record
  – including “PRIMARY COMPLETION DATE”

• Have PI RE-SUBMIT record

• Contact Elizabeth Gendel, who will help with the results entry process
FDA Fines for Late ClinicalTrials.gov Results

- FDA fines of $10,000 plus $10,000 per day thereafter
- FDA performs audits of ClinicalTrials.gov records
Public Shaming Website for Late Results

https://fdaaaa.trialstracker.net/

FDAAA 2007 is a law that requires certain clinical trials to report results. After a long wait, it effectively came into force for all trials due after January 2018. The FDA are not publicly tracking compliance. So we are, here.

Who’s sharing their clinical trial results?

- Trials reported: 831 out of 1406
- Percent reported: 59.1%
- US Govt could have imposed fines of at least $620,260,366
- Fines claimed by US Govt: $0
THANK YOU!

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