Technology Readiness Assessment

INTRODUCTION

The biggest obstacles to commercialization are often the following: poor framing of the commercial relevance of a technology (ideal use of invention, competitive advantages and novelty); an incomplete understanding of the market opportunity (e.g., patient population, clinical use, reimbursement potential); lack of a robust intellectual property (IP) position; and, insufficient preclinical data required to attract external investment for further development and adoption into the clinic.

This **Technology Readiness Assessment** document has been created for an investigator's personal use to support investigators as they consider the commercialization of their invention. Specifically, it serves to create awareness about the current stage of technology maturity and the issues that may need to be considered to translate a technology into a commercial product. The document addresses potential obstacles in the form of a scoring rubric. The investigator is not expected to have clear answers to all the questions in the rubric and is thus encouraged to reach out to their Technology Transfer Office (TTO) and other experts who will assist along this path. This document also provides an example of how to use the assessment rubric to evaluate an academic-based, early-stage cell therapy technology (stem cell-derived red blood cells) for commercial viability and future development.

GUIDE TO USING THE TECHNOLOGY READINESS ASSESSMENT

1. Each question is assigned a score of 1, 3, or 5.

2. There are three categories: Technology, Potential for Clinical Translation and Market Opportunity. For early-stage development, Technology and Market Opportunity are given the most consideration. Furthermore, each question is weighted within its category according to its importance for early stage technology commercialization.

3. Pick the most appropriate score from the drop-down menu. A total score will be generated for each section once completed. Don't be surprised if the Technology and Market Opportunity categories score individually higher than the "Potential for Clinical Translation" category. This is expected at an early stage of preclinical development. Furthermore, you may benefit from re-scoring your technology as you progress along your developmental pathway leading to an overall improvement to the scores.

4. Please see the case study below for a practical example on how to use this assessment.

5. Regardless of the score that your technology receives for each section, please make sure to contact your TTO to discuss the strengths and weaknesses that were identified by the assessment. The TTO will be able to assist you with next steps in more detail (please see the "Inventions and Working with Your Technology Transfer Office: Frequently Asked Questions" document for further information).

TECHNOLOGY READINESS ASSESSMENT

	PRIORITY	SCORE (1-5)
TECHNOLOGY		
T1. How disruptive or ground-breaking is this technology?	High	Select
T2. At what stage is the technology's development?	High	Select
T3. Have patents been filed to protect this technology?	Low	Select
T4. How technically difficult is it to conduct the next critical experiments that will substantially advance development of the technology?	Medium	Select
		TOTAL SCORE: 0 /20
POTENTIAL FOR CLINICAL TRANSLATION		
P1. Do you understand the clinical need and the required development activities to reach the clinic?	Medium	Select
P2. At what stage is the product's manufacturing?	Medium	Select
P3. Are there clear obstacles in bringing the product to patients (e.g., underdiagnosed disease, difficulty recruiting patients for clinical trials, effective and/or inexpensive current treatments, complex therapy and/or method of administration) and, if so, is there a strategy in place?	Low	Select
P4. Do you have funding to conduct the next critical experiments?	High	Select
		TOTAL SCORE: 0 /20
MARKET OPPORTUNITY		
M1. What is the size of the patient population?	High	Select
M2. Are there any companies developing similar products and how far along are they?	High	Select
M3. Have you received interest from industry/investors? Are there any obvious industry partners?	Medium	Select
M4. How attractive is this technology space (e.g., cell therapy, gene editing) likely to be to investors?	Medium	Select
		TOTAL SCORE: 0 /20

REGENERATIVE MEDICINE CASE STUDY

The case study below aims to familiarize the reader with important considerations before, or shortly after, disclosing an invention to their TTO. It describes a situation wherein an investigator is developing an early-stage, regenerative medicine-based technology in an academic laboratory. In this case, the investigator has developed an unoptimized cell therapy that can be used as a replacement for blood transfusions. The vast majority of technologies that are disclosed to the TTO fall within this developmental stage. For introductory information on considerations for technology commercialization and how to prepare for a discussion with the TTO, please see the "Early Considerations for Commercialization" and "Inventions and Working with Your Technology Transfer Office: Frequently Asked Questions" documents, respectively.

"Dr. X, a professor at Provincial University, is a recognized expert in the study of mouse erythropoiesis with over 100 primary publications. Recently, her lab developed a differentiation protocol for producing mature, enucleated red blood cells from mouse induced pluripotent stem cells (iPSCs). The *in vitro* differentiation protocol and subsequent characterization have been published in several reputable journals. Over the past few years, Dr. Y, a post-doc in the Dr. X lab, has been attempting to transition the protocol to human cells. This has been a challenging endeavor, but Dr. Y is very excited about some new results that she recently presented as a poster at an international conference. Their vision is to generate large numbers of red blood cells to be used in place of blood transfusions and they are very keen to commercialize this technology.

In brief, Dr. Y's protocol is as follows: undifferentiated iPSCs are formed into aggregates and treated with a cytokine cocktail to promote mesoderm differentiation. After four days of differentiation, a second cytokine cocktail is administered to promote development of hematopoietic progenitor cells, and at day 9-10 of differentiation $CD34^+$ cells are isolated by cell sorting. The resulting cells are then plated into erythroid differentiation media for a further 18 days for erythroid maturation. The iPSC differentiation is currently initiated in a single 6-well plate, and 500-fold-expansion is observed during erythroid differentiation from isolated $CD34^+$ cells. It is estimated that ~10^8 cells could be generated during each production run. Terminally differentiated cells have been analyzed by flow cytometry and are observed to be 44-58% $CD71^+$/$CD235^+$.

Dr. X spoke with a colleague with clinical expertise in this area who said that this technology could address a significant clinical unmet need for blood replacement products. He also told Dr. X that there are 300 million blood transfusion procedures conducted annually and that demand was steadily increasing due to a rise in the number of blood disorders and surgical procedures.

Drs. X and Y are very excited about the potential of this technology and are interested in filing a patent application on this work. They also have an ongoing grant to pursue this research for the next year only. They intend to reach out to their institutionally-affiliated TTO to determine the commercial potential, patentability and next steps in the commercialization process for their technology. In parallel, Drs. X and Y discovered the "Technology Readiness Assessment" document online. Although this assessment is not part of the commercialization process laid out by their institution, nor required by their TTO, they intend to run their technology through this optional assessment to understand its commercial viability and potential for future development."

ASSESSMENT OF CASE STUDY TECHNOLOGY

Please refer to our justification for these scores in the next section

	PRIORITY	SCORE (1-5)
TECHNOLOGY		
T1. How disruptive or ground-breaking is this technology?	High	**3: Novel technology for an existing or new market**
T2. At what stage is the technology's development?	High	**5: Data generated for in vivo model and/or human cells in vitro**
T3. Have patents been filed to protect this technology?	Low	**1: Patent application has not been filed**
T4. How technically difficult is it to conduct the next critical experiments that will substantially advance development of the technology?	Medium	**3: Moderate; some risk**
		TOTAL SCORE: 12/20
POTENTIAL FOR CLINICAL TRANSLATION		
P1. Do you understand the clinical need and the required development activities to reach the clinic?	Medium	**3: Some understanding; previous engagement with clinicians**
P2. At what stage is the product's manufacturing?	Medium	**1: Synthesized at lab scale**
P3. Are there clear obstacles in bringing the product to patients (e.g., underdiagnosed disease, difficulty recruiting patients for clinical trials, effective and/or inexpensive current treatments, complex therapy and/or method of administration) and, if so, is there a strategy in place?	Low	**1: >1 barrier, no strategy**
P4. Do you have funding to conduct the next critical experiments?	High	**3: Limited funding to conduct some experiments**
		TOTAL SCORE: 8/20
MARKET OPPORTUNITY		
M1. What is the size of the patient population?	High	**5: Large market (>500k>1M patients/yr globally)**
M2. Are there any companies developing similar products and how far along are they?	High	**3: Competitors in preclinical development**
M3. Have you received interest from industry/investors? Are there any obvious industry partners?	Medium	**1: No interest**
M4. How attractive is this technology space (e.g., cell therapy, gene editing) likely to be to investors?	Medium	**5: Attractive - viable comparables & investors are interested in field**
		TOTAL SCORE: 14/20

SCORING JUSTIFICATION

Technology

T1. Score "3": At the time of scoring, Dr. X believes that this product can only be used as a replacement blood product for transfusions. However, after a discussion with her TTO she determines it may have other applications such as: i) a novel drug delivery method; ii) edited/modified therapeutic red blood cells; and, iii) a screening or research tool for studying gene regulation. These additional future applications could increase the score to "5" since the technology may represent a platform technology if data can be generated for these other applications.

T2. Score "5": The inventors have tested CD34+ human cells *in vitro*.

T3. Score "1": The inventors are interested in filing a patent application.

T4. Score "3": Next experimental steps will require scale up of cell production (this might include growing cells in a bioreactor and thus possible amendments to the existing protocol) and higher purity of the terminally differentiated population.

Potential for Clinical Translation

P1. Score "3": The inventors spoke with a clinician colleague and now have some understanding of the limitations of current transfusion approaches and future clinical considerations.

P2. Score "1": The inventors have generated terminally-differentiated cells although at low viability. Scale-up has not been completed and there is a recognition of both expansion and the requirement of an immense number of total red blood cells for transfusion applications.

P3. Score "1": The inventors need to demonstrate a strong comparative benefit to outweigh decades of clinical experience with blood donation products (i.e., the current treatment) as these are relatively inexpensive and straightforward compared to the potential cost and complexity of the new approach (based on currently approved cell therapies). In addition, there are competing cell therapy technologies such as those in development by Rubius Therapeutics.

P4. Score "3": Inventors currently have a research grant that allows them to pursue this work for the next year.

Market Opportunity

M1. Score "5": An industrial colleague indicated that the global number of blood transfusion procedures is 300 million annually.

M2. Score "3": Dr. X did a quick Google search and found that most competitors are only in preclinical development (e.g., Rubius Therapeutics).

M3. Score "1": The inventors did not receive any prior interest from industry.

M4. Score "5": There is a high unmet need for a replacement blood product and the potential market is large. Thus, the technology should be attractive to investors.

CONCLUSION

Dr. X was pleased to find that her technology scored highly in the "Technology Readiness" (60%) and "Market Opportunity" (70%) sections. However, she noted that it scored low in the "Potential for Clinical Translation" (33%) category. This is not a major concern as optimization of her technology, and a better understanding of the path to market, can improve the score for this category in the future. After using the rubric, she followed the next steps recommended by her TTO (and broadly described in the "*Inventions and Working with Your Technology Transfer Office: Frequently Asked Questions*" document). Her TTO conducted a brief review of the market and major competitive players in this space. They also connected her with a licensed patent agent who confirmed that the technology could be used for multiple product concepts (as described above), thereby increasing its market applicability as a platform technology.

One area of concern was a need to optimize for future manufacturing. She spoke with her TTO and they recommended discussing these issues with cell and gene therapy manufacturing experts.

Future points for consideration:

- Demonstrate improved cell purity of mature cells and address scalability concerns; cell numbers are estimates
- Demonstrate functional data showing that generated cells are phenotypic erythrocytes
- Determine *in vivo* persistence in comparison to donor cells
- Understand the potential cost of proposed therapy and regulatory approvals vs. conventional blood products

This is the start of the commercialization journey and you will have many partners along the way, such as your TTO, funding networks like Stem Cell Network, and manufacturing experts like CCRM. These partners will work closely with investigators to help accelerate the development of regenerative medicine-based technologies and cell and gene therapies.

APPENDIX

The following section expands on the technology assessment criteria presented in the scorecard. Its goal is to provide additional explanation and links that might be useful for the inventor to learn and consider in the context of a technology commercialization plan.

Commercialization: The process of taking an invention or scientific discovery to the market.

Competitive advantage: An invention's unique selling proposition i.e. how is this product better than what currently exists on the market or in development.

Good Manufacturing Practices (GMP) manufacturing: A system of practices that ensure that therapeutic products are controlled and produced consistently in accordance to quality standards.

End-user: The person who ultimately uses a product or service (e.g., medical doctor, surgeon, patient).

Intellectual Property: Creations of the mind, such as inventions, literary and artistic works, designs and symbols, and names and images used in commerce.

Investor: An individual or entity that puts money into another entity, such as a business, for a financial return based on key investment assessment criteria.

License: A type of legal agreement from a patent owner that provides a third party with the rights to use the patented technology commercially.

Patent: A time-limited monopoly on rights to an invention in a particular jurisdiction (e.g., rights to make, use, or sell a technology). They are usually filed with the help from an institution's TTO and/or a patent agent. They require a full description of the invention and detailed claims that define the boundaries of the protection to be granted.

Platform technology: A technology that can generate multiple products or therapies for a number of different diseases. These are generally more appealing to investors as this may generate higher revenue once the company is established.

Target market: The people who will use your product or service.

DISCLAIMER