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Stem Cell Therapy for Muscle Regeneration
Business Executive Search

A potential University of Minnesota (UMN) startup focused on pluripotent stem cell therapy for muscle diseases, including Duchenne muscular dystrophy (DMD) is seeking a business exec to co-found the business with the UMN and the research team.

Industry sector(s)
Biotech

Inventor/Founders:
Dr. Rita Perlingeiro
Dr. Michael Kyba

Company status:
Generation of cGMP product is currently underway, with testing expected in winter 2021 and IND filing in 2022

Overview
Induced pluripotent stem cells (iPSC) have tremendous potential to treat degenerative conditions and genetic diseases. Research at the UMN has shown the potential for stem cell-based therapy, comprising human skeletal myogenic progenitors derived from pluripotent stem cells, to replenish diseased muscle with normal functional muscle fibers as well as muscle stem cells enabling long-term muscle maintenance. This treatment has the promise of regenerating muscle lost to a variety of conditions, including various devastating muscular dystrophies, such as DMD. The research performed to date provides extensive pre-clinical validation for a method to generate engraftable skeletal myogenic progenitors from pluripotent stem cells, and the implementation of this method under cGMP conditions. These myogenic progenitors, when transplanted into dystrophic mice, produce large quantities of functional skeletal muscle tissue that incorporates normally into the host muscle. Importantly, a fraction of transplanted cells remain mononuclear, and display key features of skeletal muscle stem cells, including satellite cell localization, response to re-injury, and contribution to muscle regeneration in secondary transplantation assays. The result is a technology that comprises a cell therapy for muscle disease that may rebuild functional skeletal muscle and provide robust support for future damage.

Progress, milestones, or goals
Over the last decade the research team has demonstrated extensive proof-of-concept. Recently the team has optimized the manufacture of these cells under conditions that are compatible with clinical application, including addressing aspects related to purification, scalability, safety, and manufacturing. The program had a successful and productive Pre-Investigational New Drug (Pre-IND) Consultation with the Food and Drug Administration (FDA) in 2017. Following extensive studies with research grade iPSC-derived myogenic progenitors, the team, with support from Duchenne UK, manufactured PSC-derived myogenic progenitors (MyoPAXon) under cGMP conditions and is using them for preclinical studies (biodistribution and safety) using murine recipients. The early

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results from these studies are promising and according to our pre-IND interaction, these will enable the First-in-Human Phase 1 Safety/Dose Escalation Trial of MyoPaxon for DMD.

Business executive activities, tasks, and responsibilities

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- Develop the business plan and business model with the help of the UMN research team and the Discovery Launchpad resources
- Develop and implement a financing strategy and raise capital as needed
- Operate and scale the business, drive the business to success
- Review financial and non-financial reports to devise solutions or improvements
- Lead and motivate subordinates to advance employee engagement develop a high performing managerial team
- Maintain a deep knowledge of market and industry trends
- Establish a board of governance

Business executive skills and abilities

- Have the relevant experience in the biotech and specifically stem cell therapy
- Have an entrepreneurial mindset with outstanding leadership skills
- Leadership skills to pull together the resources to operate the business
- Experience and connections to develop successful strategic partnerships
- Be familiar with diverse business functions such as marketing, sales, finance, human resources, etc.
- Create, communicate, and implement the vision for the company
- Be effective in communicating and have strong public speaking skills
- Possess outstanding organizational and time management skills
- Ability to analyze problematic situations and occurrences and provide solutions to ensure company survival and growth
- Excellent interpersonal and leadership skills

Qualities preferred

- Experience in successful startup leadership
- Experience in raising early-stage capital
- Proven track record of building successful products and high performing teams from the ground up

IP Status:

UMN 20140040
Gene Targeting Methods and Tools
US Patent 9,850,497 issued

UMN 20160306
Identification of Cell Surface Markers for the Isolation of Human Myogenic Stem/Progenitor Cells
US Patent Publication 2019/0352611 pending

UMN 20180079
Enhanced differentiation and maturation of pluripotent stem cell-derived myogenic cells
US Patent Publication 2021/0171911 pending

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Compensation

- Compensation for this role will be determined by the founders of the company. Candidates for this position should have considerable runway.
- The University of Minnesota and the Technology Commercialization Office will not provide nor be responsible for any cash or non-cash compensation for this role.
- The primary compensation may be consistent with typical startup compensation strategies including an equity position in the company in the form of founder's shares.
- Percentage of ownership, vesting timelines and other details of an offer will be negotiated with the other founding team members.
- All costs incurred in this position will be your responsibility.
- Expect any cash or monetary compensation to start below market. The performance, success and trajectory of the company may influence an increase or acceleration of benefits.
- It will be expected that the person pursuing this role will sign an MOU that includes, at a minimum, the items above.

If you are interested in this opportunity, please contact Russ Straate at rstraate@umn.edu