



## Induced Pluripotent Stem Cell Technology: A New Era in Drug Discovery

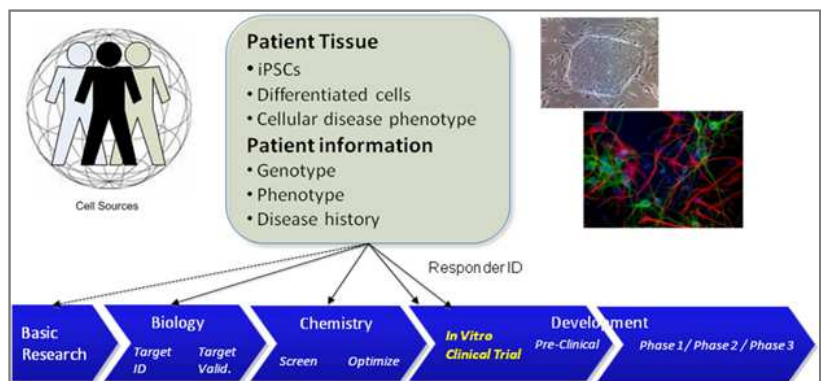
Scientists and clinicians agree that human stem cells hold enormous potential to revolutionize the treatment of diseases. iPierian is developing cellular reprogramming and differentiation technologies that apply the power of pluripotent stem cells to transform drug discovery and enable the promise of regenerative medicine. By generating pluripotent stem cells from individual patients, these technologies will unleash the potential to study and battle diseases in ways never before possible.

iPierian is the first company to focus the power of iPSC technology to revolutionize the drug discovery process by producing iPSC cells derived from patients to create truly disease-relevant model systems. These systems can be used for the discovery of new drugs and ultimately for treating diseases more safely and effectively.

iPierian's efforts are centered on applying its technology to diseases for which there are poor in vivo and in vitro models and limited therapeutic treatments to date. iPierian's proprietary programs are initially focused on neurological disorders, including Parkinson's disease, Huntington's disease, spinal muscular atrophy (SMA) and amyotrophic lateral sclerosis (ALS or Lou Gehrig's disease). These conditions have limited therapeutic treatments, and scientists have demonstrated the ability to differentiate the affected cell types in these disorders. iPierian is also working on calcific aortic valve disease through its collaboration with the Gladstone Institute of Cardiovascular Disease and iPierian has initiated programs to address metabolic diseases including type 2 diabetes and obesity.

## iPierian's Approach to Cellular Reprogramming and Directed Differentiation

iPierian is focused on the application of cellular reprogramming, including the scientific breakthrough of induced pluripotent stem cell (iPSC) technology as well as directed differentiation methods. iPierian's technology involves reprogramming somatic cells into a pluripotent state. Because iPSC cells have stem-cell-like properties and have the potential to differentiate into a variety of cell types, iPSC cells are considered to be of great potential for disease research, drug discovery and development. In addition, iPSC cells avoid the ethical issues specific to human embryos and human embryonic stem (hES) cells.



Based in part on its patent filings for the creation of human iPSC cells, including iPierian's granted patent in the United Kingdom, iPierian's technology involves creating disease-relevant model systems from reprogrammed cells obtained from skin cells or other non-embryonic sources. iPierian's approach places the patient at the forefront of the drug discovery process.

The ability to study patient-specific, differentiated iPSC cells in vitro represents a true paradigm shift in drug discovery. iPierian's state-of-the-art pluripotent stem cell generation capabilities will enable the identification of compounds with a higher probability of success in clinical development. iPierian's directed differentiation capabilities also can be applied to assess the selectivity and toxicity of specific compounds in cell types of interest, and to identify drug-responders prior to

going to the clinic – such as which phenotypic and genotypic patient profiles will respond to a particular drug – both of which could accelerate and reduce the clinical costs of drug development.

## **Partnering to Advance the Technology**

iPierian plans to apply this revolutionary technology in proprietary programs and leverage it in collaborations with pharmaceutical and biotechnology industry partners. Current collaborators include the Gladstone Institute of Cardiovascular Disease and Dr. Deepak Srivastava, the institute's director, in the area of calcific aortic valve disease (CAVD) and the laboratory of Dr. Shinya Yamanaka of Kyoto University to develop and improve methods of deriving iPSC cell lines in order to advance iPSC technology.

## **iPierian's Management Team and Scientific Leadership**

iPierian has built one of the most qualified teams of management and stem cell scientists in the world, combining the expertise of the founding scientists of what was formerly iZumi Bio with internationally-renowned scientists from top institutions such as the Harvard Stem Cell Institute and University of California San Francisco among others.

### **Management Team**

- John Walker, Chief Executive Officer
- Dr. Michael Venuti, President and Chief Scientific Officer
- Dr. Berta Strulovici, Chief Technical Officer
- Dr. Dushyant Pathak, Vice President Business Development
- Julie McDonnell, Vice President Legal Affairs
- Dr. Matthew Plunkett, Vice President and Chief Financial Officer

### **Board of Directors**

- Dr. Corey Goodman, Chairman of the Board of Directors
- Dr. Ashley Dombkowski, Managing Director, MPM Capital
- Bob Higgins, General Partner, Highland Capital
- Dr. Beth Seidenberg, Partner, Kleiner Perkins Caufield & Byers
- John Walker, Chief Executive Officer, iPierian

### **Scientific Advisory Board**

- Dr. George Daley, Co-Chair, Children's Hospital, Dana Farber Cancer Institute, Harvard Medical School
- Dr. Deepak Srivastava, Co-Chair, Gladstone Institute of Cardiovascular Disease, University of California, San Francisco (UCSF)
- Dr. Douglas Melton, Harvard Stem Cell Institute, Howard Hughes Medical Institute, Harvard University
- Dr. Corey Goodman, National Academy of Sciences
- Dr. Lee Rubin, Harvard Stem Cell Institute
- Dr. Lennart Mucke, Gladstone Institute of Neurological Disease, UCSF
- Dr. Benoit Bruneau, Gladstone Institute of Cardiovascular Disease, UCSF
- Dr. Chad Cowan, Harvard Medical School, Stowers Medical Institute
- Dr. Kevin Egan, Howard Hughes Medical Institute, Harvard University, Harvard Stem Cell Institute, Stowers Medical Institute
- Dr. Matthias Hebrok, Diabetes Center at UCSF
- Dr. Konrad Hochedlinger, Harvard Medical School, Harvard Stem Cell Institute, Massachusetts General Hospital
- Dr. Amy Wagers, Joslin Diabetes Center, Harvard University, Harvard Stem Cell Institute