

MARKET REPORT



Induced Pluripotent Stem Cells In Research & Drug Discovery Trends 2015

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Induced Pluripotent Stem Cells In Research & Drug Discovery Trends 2015

HTStec's Induced Pluripotent Stem Cells In Research & Drug Discovery Trends 2015 report was published on 1 December 2015. This 71 page market report summarizes the results of HTStec's industry-wide global web-based benchmarking survey on induced pluripotent stem cells (iPSC) in research and drug discovery carried out in November 2015. The study was initiated by HTStec as part of its ongoing tracking of emerging life science markets and in some aspects updates our previous report on the subject (December 2012). The questionnaire was compiled to meet the needs and interests of the stem cell vendor community. The main objectives of this global benchmarking study were to understand current interest, practices and preferences in carrying out research on stem cells and stem cell-derived cells (particularly iPSC) as well as their utilization in different areas of drug discovery and development. The findings of this research study comprise important feedback for vendors of iPSC and iPSC-related products in order to understand customer needs and focus future product development efforts. The report is based on 210 responses mainly from University & Research Institute, Pharma and Other Screening Lab segments in Europe and North America.

Executive Summary

- This market report summarizes the results of HTStec's industry-wide global web-based benchmarking survey on induced pluripotent stem cells (iPSC) in research and drug discovery carried out in November 2015.
- The main objectives of this global benchmarking study were to understand current interest, practices and preferences in carrying out research on stem cells and stem cell-derived cells (particularly iPSC) as well as their utilization in different areas of drug discovery and development.
- The study was initiated by HTStec as part of its ongoing tracking of emerging life science markets and in some aspects updates our previous report on the subject (December 2012). The questionnaire was compiled to meet the needs and interests of the stem cell vendor community.
- The findings of this research study comprise important feedback for vendors of iPSC and iPSC-related products in order to understand customer needs and focus future product development efforts.
- The survey looked at the following aspects of iPSC and/or iPSC-derived cells in research and drug discovery as practiced today (2015) and in some cases as predicted for the future (2017): % of stem cell work that involves different stem cell types; obstacles limiting work with stem cell-derived cells; implementation of stem cell differentiation protocols; cell lineages into which respondents are attempting to differentiate stem cells; work involving iPSC; source of iPSC or iPSC-derived cells; main barriers to sourcing iPSC needs commercially; work involving the generation of iPSC; steps in iPSC workflow undertaken; annual budget for consumables to generate iPSC; budget breakdown into research areas; most purchased from suppliers of consumables used to generate iPSC; purchasing of commercial iPSC; main suppliers of commercial iPSC used; most important factors when choosing a supplier of commercial iPSC; annual budget for purchasing commercial iPSC and breakdown into different cell types; factors that most influence purchasing of iPSC; iPSC-derived cell types of interest that are not commercially available; commercial iPSC-derived cell types of major interest; undertaking toxicology/safety testing using iPSC; % of toxicology/safety testing assays using different cell types; main source of iPSC used in toxicology/safety testing assays; relative use of iPSC-derived cell types in toxicology/safety testing assays; number of compounds tested per year for safety/toxicology applications using iPSC; assay technologies applied to toxicology/safety testing using iPSC; undertaking phenotypic screening assays using iPSC; % of phenotypic screening assays using different cell types; main source of iPSC used in phenotypic screening assays; number of data points (microplate wells) screened per year in phenotypic assays using iPSC; undertaking disease modeling using iPSC; preferred approach to disease modeling; type of disease models being developed; implementation of iPSC-derived cells into existing drug discovery-related assays; drug discovery-related assays which might advantageously be complemented or replaced with iPSC-derived cells; other application areas of iPSC; and likelihood of using any iPSC- or ESC-related services and customization.
- The main questionnaire consisted of 43 multi-choice questions. The exact number presented to respondents

depended upon their specific use and interests of stem cells. In addition, there were 6 questions related solely to survey demographics.

- The survey collected 210 validated responses, of these 64% (135 out of 210) completed the survey.
- Survey responses were geographically split: 50% Europe, 31% North America, 8% Asia (excluding Japan), 7% Japan, 2% China; and 2% Rest of World.
- Survey respondents were drawn from persons or groups using or interested in using stem cells in life science research and drug discovery applications.
- Respondents came from 51 University; 31 Large Pharma; 29 Research Institute; 26 Biotech Company; 25 Contract Research Organization; 13 Medium-Small Pharma; 8 Government Lab; 7 Hospital/Clinic/Medical School; 5 Biopharma; 5 Not-For-Profit Research Center; and 2 Academic Screening Center.
- Most survey respondents had a senior job role or position which was in descending order: 26 research scientists; 22 professors/assistant professors; 18 senior scientists/researchers; 18 principal investigators; 11 post-docs; 10 other roles; 9 section/group leaders; 8 directors; 4 graduate/PhD students; 3 lab managers; 3 department heads; and 1 vice president.
- Survey results were expressed as an average of all survey respondents. In addition, where appropriate the data was fully reanalyzed after sub-division into the following 5 survey groups: 1) Pharma; 2) Other Screening Labs; 3) University & Research Institute; 4) Europe; and 5) North America.
- The majority (80%) of survey respondents were currently using stem cells.
- iPSC or iPSC-derived cells were the most investigated stem cell type.
- Cost of commercially available cells was rated the main obstacle limiting work with stem cell-derived cells.
- 34% of survey respondents were currently implementing in house stem cell differentiation protocols.
- The cell lineages into which most were attempting to differentiate stem cells were cardiac muscle cells, brain/neuronal/glia cells and hepatocytes.
- 74% of survey respondents were currently working with iPSC and iPSC-derived cells.
- A median of 11-25% of iPSC and 51-75% of iPSC-derived cells are sourced commercially today.
- Cost was rated the main barrier to sourcing iPSC or iPSC-derived cell needs commercially.
- 31% of survey respondents were currently involved in the generation of iPSC or iPSC-derived cells.
- The step in the iPSC workflow most undertaken by those generating iPSC or iPSC-derived cells was cell analysis/characterization/validation.
- The median annual budget allocated for spending on iPSC and iPSC-derived cell generation consumables Today (2015) was \$50K-\$100K.
- A bottom-up model developed around respondent's budget estimated the market for iPSC generation consumables used in drug discovery labs to be around \$44M in 2015.
- The most purchased from supplier of iPSC generation consumables was Thermo Fisher Scientific.
- 70% of survey respondents have purchased commercial iPSC or iPSC-derived cells.
- The main suppliers of commercial iPSC and iPSC-derived cells were Cellular Dynamics International and Axiogenesis.
- Level of validation of cells (including publications) was rated the most important factor in choosing a supplier

of commercial iPSC or iPSC-derived cells.

- The median annual budget allocated for purchasing commercial iPSC and iPSC-derived cells today (2015) was \$10K-\$25K.
- A bottom-up model developed around respondent's budget estimated the market for commercial iPSC purchased for drug discovery-related assays to be around \$45M in 2015. Cardiomyocyte sales make up the biggest proportion of this market.
- Level of characterization/validation of cell type was rated the factor that will most influence expected purchasing on iPSC and iPSC-derived cells.
- 38% of survey respondents were aware of iPSC-derived cell types of interest that are not commercially available, and provided details of the iPSC types lacking.
- The commercially available iPSC-derived cell type of most interest was cardiomyocytes (ventricular-enriched).
- 38% of survey respondents were currently undertaking toxicology/safety testing in house using iPSC-derived cells.
- The current (2015) breakdown of toxicology/safety testing assays between different cell types was 41% stem cells, 39% cell lines and 20% primary cells.
- The main source of iPSC-derived cells used in toxicology/safety testing assays was commercial off-the-shelf cell aliquots.
- The iPSC-derived cell type most used in toxicology/safety testing assays today (2015) were cardiomyocytes.
- A median of 1-25 compounds were tested per year for toxicology/safety applications using iPSC-derived cells today (2015).
- Calcium transients/voltage sensitive dye assays were rated the assay technology survey respondents were most interested in applying to toxicology/safety testing using iPSC-derived cells.
- 41% of survey respondents were currently undertaking phenotypic screening assays using iPSC-derived cells.
- The current (2015) breakdown of phenotypic screening assays between different cell types was 55% stem cells, 24% cell lines and 21% primary cells.
- The main source of iPSC-derived cells used in phenotypic screening assays was commercial off-the-shelf cell aliquots.
- A median of 1K-5K data points (microplate wells) were screened per year in phenotypic assay applications using iPSC-derived cells today (2015).
- 35% of survey respondents were undertaking disease modeling in house using iPSC-derived cells.
- Selection of donor material (genotype selection) was the preferred approach to disease modeling using iPSC-derived cells.
- The most reported disease model being developed using iPSC-derived cells was Parkinson's disease.
- Those survey respondents that have implemented iPSC-derived cells into existing drug discovery related assays provided brief details of the assays.
- Those survey respondents that could think of drug-discovery related assays which might be advantageously complemented or replaced with iPSC-derived cells provided brief details of the assays.
- Interest in other application areas of iPSC or iPSC-derived cells was greatest for investigation of stem cells in regenerative medicine/tissue engineering/cellular therapies and molecular pathway analysis.

- Custom generation of specific iPSC or ESC-derived cell types was rated the service or customization involving iPSC or ESC survey respondents were most likely to use over the coming years (up to 2017).
- The full report provides the data, details of the breakdown of the responses for each question, its segmentation and the estimates for the future (2017). It also highlights a few interesting differences between the survey groups, particularly Pharma and Other Screening Labs versus University & Research Institute

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HTStec (formerly HTS Technologies) is an independent consultancy that was founded by Dr. John Comley in spring 2002. HTStec is focused on providing informed opinion and market research on those enabling and emerging technologies that underpin high throughput screening (HTS) today. HTStec works with clients, drawn mainly from those companies that are developing novel liquid handling and detection instruments, laboratory automation, assay reagents and platform technologies, to help them maximise the market potential of their developments and gain the competitive edge through better understanding of the latest requirements of customers working in HTS laboratories.

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