MARKET REPORT

Phenotypic Drug Discovery Trends 2015

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Phenotypic Drug Discovery Trends 2015

HTStec's Phenotypic Drug Discovery Trends 2015 report was published on 21 September 2015. This 60 page market report summarizes the results of HTStec’s industry-wide global web-based benchmarking survey on phenotypic drug discovery (PDD) carried out in August 2015. The survey was initiated by HTStec as part of its tracking of this emerging life science marketplace and to update HTStec’s previous report on the subject (published March 2013). The questionnaire was compiled by HTStec and attempts to meet the needs, requirements and interests of vendors hoping to address this space. The main objectives were to understand the latest thinking, current practices and preferences in PDD and future requirements for phenotypic primary screening. The report is based on 57 responses from University Labs, Pharma and Biotech mainly in Europe and North America.

Executive Summary

• This market report summarizes the results of HTStec’s industry-wide global web-based benchmarking survey on phenotypic drug discovery (PDD) carried out in August 2015.

• The survey was initiated by HTStec as part of its tracking of this emerging life science marketplace and to update HTStec’s previous report on the subject (published March 2013).

• The questionnaire was compiled by HTStec and attempts to meet the needs, requirements and interests of vendors hoping to address this space. The main objectives were to understand the latest thinking, current practices and preferences in PDD and future requirements for phenotypic primary screening.

• The survey looked at the following aspects of PDD, as practiced today (2015) and in a few cases as predicted for the future (2017): current level of deployment (actual use); what features most highly in the understanding of PDD; definition of a phenotypic screening assay; assay types that are readily amenable to the target agnostic/biological mimetic approach; key diseases/therapeutic area(s) deploying phenotypic screening assays; main motivators for wanting to adopt PDD; key challenges of doing PDD; obstacles which would most limit the adoption of phenotypic screening assays today; number of FTE devoted in house to PPD; the number of primary phenotypic screens and wells per screen; the average cost per well of phenotypic screening assays; cell types most suited for phenotypic screening studies; biological systems currently used for PDD; expectations from phenotypic screening; success of PDD efforts to date; PPD project milestones achieved to date without knowledge of the target; where phenotypic assay approaches are best suited to be used; approaches used in pathway and target deconvolution; level of agreement with some statements about PDD; prerequisites for starting PDD; is there a critical path for PDD; type and size of compound libraries screened in PDD; does PDD require new types of compounds libraries; annual reagent and consumables budget for all work related to phenotypic assays and it breakdown into component parts; assay technologies and instrument platforms currently used in PDD; assay reagent and instrument platform vendors seen as key players in PPD; and any unmet needs in PDD or new tools are required to drive the investigation of phenotypic screening assays.

• The main questionnaire consisted of 24 multi-choice questions and 6 open-ended questions. In addition, there were 6 questions related solely to survey demographics.

• The survey collected 57 responses, of these 58% provided comprehensive input.

• Responses were geographically split: 44% North America; 32% Europe; 14% Asia (excluding Japan & China); 5% Japan; and 2% China.

• Survey respondents were drawn from persons or groups undertaking PDD and phenotypic primary screening. In addition feedback was sought from those interested in PPD and considering future investigation in this area.

• Respondents represented 15 University; 13 Large Pharma; 7 Academic Screening Centers; 7 Research Institute; 6 Medium-Small Pharma; 3 Biotech Company; 3 Hospital/Clinic/Medical School; 1 Government Lab; 1 Not-For-Profit Research Center; and 1 Contract Research Organizations.

• Most survey respondents had a senior job role or position which was in descending order: 11 principal

investigators; 10 professors/assistant professors; 8 senior scientists/researchers; 7 directors; 6 section/ group leaders; 4 department heads; 3 research scientists; 2 others; 2 vice presidents; 2 post-docs; 1 lab manager; and 1 graduate/PhD student.

• Respondents represented the followings main activities: 18 with a combination of drug discovery areas; 12 primary screening (HTS); 10 basic research; 5 assay development; 5 applied research; 3 other; 2 hits-toleads (lead optimization); 1 leads-to-candidate (ADME Tox/preclinical research); and 1 therapeutic areas (target ID/validation).

• Survey results were expressed as an average of all survey respondents. In addition, where appropriate the data was reanalysed after sub-division into the following 5 survey groups: 1) Pharma; 2) Biotech; 3) Academic Research; 4) Europe; and 5) North America.

• 90% of respondents were currently using PDD approaches (to varying extents) today. The remaining 10% were planning future investigation.

• Captures bio content (targets presented in a physiological context) was ranked the most important features in understanding PDD.

• The assay types used in a TTD application that are most amenable to the target agnostic/biological mimetic approach associated with PDD were assays involving primary cell cultures or co-cultures of primary cells.

• The majority of respondents were targeting PDD within the oncology therapeutic area.

• Allows for discovery of unexpected biology was ranked the main motivator for wanting to adopt PDD.

• Mentalities are still target-orientated was rated as the key challenge (major issue) of doing PDD.

• Understanding the biological endpoints needed was rated the main obstacle limiting adoption of phenotypic screening assays.

• A median of 3 FTE’s were allocated to enable/support in house PDD research (investigation and screening) in 2015.

• A median of 3 different phenotypic projects/programs were undertaken in 2015.

• A median of 3 phenotypic primary screens each with 10K-25K wells were done per year in 2015.

• The median cost of a phenotypic screening assay undertaken in 2015 was $0.75-$1 per well.

• Human primary cells were ranked the cell type most suited (relevant) for phenotypic screening studies.

• Cell line monocultures were the biological system currently most used to investigate PDD.

• Novel MOA which is differentiated from the standard of care and to understand the functional responses were both equally rated as most likely (desired) outcomes for phenotypic screening.

• The median success rate for phenotypic primary screening in 2015 was 50%.

• SAR observed in phenotypic assay system was the PDD project milestone most achieved to date without knowledge of the target.

• Suitability for the identification of novel therapeutically active molecules for drug discovery was ranked the most important use of phenotypic assays.

• Signal pathway activity mapping methods were ranked the target deconvolution approach most used for the identification of phenotypic activities today.

• Respondent’s level of agreement with certain statements about PDD was recorded.

• Most respondents think there is a critical path for PDD and suggested some of the steps involved.

• Annotated compounds (compounds with known targets/mechanisms) was rated the type of compound collection that should be most screened in PDD.

• The median size of compound libraries most evaluated for PDD today (2015) was 10K-50K compounds.

• Most respondents don't know enough to comment if PDD requires new types or more diverse compound libraries.

• A median budget of $50K-$100K/lab was allocated for phenotypic assay reagents and consumables in 2015, with the greatest share allocated to assay specific reagents.

• A bottom-up model developed around respondent's % use of phenotypic screens and their annual budget for phenotypic assay reagents and consumables estimated the global market to be around $165M in 2015.

• The most preferred key vendors of assay reagents used in PDD were Thermo Scientific, Cell Signaling Technology and Promega.

• The most preferred key vendors of instrument platforms used in PDD were PerkinElmer, Molecular Devices and Thermo Scientific.

• Respondent's feedback on the following open-ended questions were documented: 1) What is understood by PDD, how does it differ from target-based discovery? 2) What defines a phenotypic screening assay? 3) What are the biggest limitations (obstacles) in using phenotypic screening assays today? 4) What are the prerequisites for starting PDD? 5) What assay technologies and instrument platforms are preferentially used for PDD? and, 6) What are the biggest unmet needs in PDD and what new tools are required to drive the investigation of phenotypic screening assays?

• The full report provides the data, details of the breakdown of the responses to each question, its segmentation, estimates for the future (2017) and CAGR estimates. It also highlights some interesting differences between the survey groups.

Additional Details

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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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