

# MARKET REPORT



## CRISPR/Cas 9 Gene Editing In Drug Discovery Trends 2016

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# CRISPR/Cas 9 Gene Editing In Drug Discovery Trends 2016

HTStec's CRISPR/Cas 9 Gene Editing In Drug Discovery Trends 2016 report was published on 2 March 2016. This 55 page market report summarizes the results of HTStec's industry-wide global web-based benchmarking survey on CRISPR/Cas 9 gene editing in drug discovery carried out in February 2016. The study was initiated by HTStec as part of its tracking of emerging life science marketplaces. The questionnaire was compiled to meet the needs, requirements and interests of the gene editing reagents vendor community. The objective was to comprehensively document the current use of CRISPR (clustered regularly interspaced short palindromic repeats) and the associated nuclease Cas 9 in gene editing in drug discovery applications and to understand its future impact. The report is based on 96 responses drawn from persons or groups interested in the gene editing, and undertaking or planning future investigation of CRISPR/Cas 9 in drug discovery related applications. Respondents came mainly from University & Research Institute, Biotech and Pharma Labs in Europe and North America.

## Executive Summary

- This market report summarizes the results of HTStec's industry-wide global web-based benchmarking survey on CRISPR/Cas 9 gene editing in drug discovery carried out in February 2016.
- The survey was initiated by HTStec as part of its tracking of emerging life science marketplaces.
- The questionnaire was compiled to meet the needs, requirements and interests of the gene editing reagents vendor community. The objective was to comprehensively document the current use of CRISPR (clustered regularly interspaced short palindromic repeats) and the associated nuclease Cas 9 in gene editing in drug discovery applications and to understand its future impact.
- Equal emphasis was given to soliciting opinion from all areas and organisations where CRISPR/Cas 9 gene editing is being utilised as part of drug discovery efforts.
- The survey looked at the following aspects of CRISPR/Cas 9 gene editing technology as practiced today (2016) and in some cases as predicted for the future (2018): current experience of gene editing; when first started using gene editing; extent utilizing in drug discovery; number of FTE working in respondent's organisation on gene editing; key diseases or research areas using gene editing; what respondents want to achieve with gene editing; genomes being targeted with gene editing; main objectives of gene editing efforts in drug discovery; advantages of CRISPR/Cas 9 gene editing technology; potential benefits of CRISPR/Cas 9 in drug discovery respondents most want to exploit; most relevant features of CRISPR/Cas 9 gene editing technology to respondent's research effort; bioinformatics tools used to design guide RNAs (sgRNAs); how CRISPR/Cas 9 components are delivered into cells; methods used for CRISPR experiments; use of different cell types in CRISPR research; percentage efficiency of gene editing typically achieved; downstream analysis techniques used to validate your CRISPR/Cas 9 gene editing; number of sgRNAs designed per gene targeted; use of multiplexed gene edits; use of CRISPR libraries for target validation or drug mechanism studies and which type of libraries preferred; cellular response endpoints of greatest value in the functional screening of CRISPR libraries; number of different CRISPR/Cas 9 gene editing projects attempted per year; budget for purchasing CRISPR/Cas 9 gene editing related reagents and breakdown into components; supplier/vendor that first comes to mind when thinking of CRISPR/Cas 9 gene editing related reagents; suppliers where respondents currently purchase the majority of their CRISPR/Cas 9 gene editing related reagents; what most limits work on CRISPR/Cas 9 gene editing today; and any unmet needs that exist in CRISPR/Cas 9 gene editing related to drug discovery today.
- The main questionnaire consisted of 29 multi-choice questions and 2 open-ended question. In addition, there were 6 questions related solely to survey demographics.
- The survey collected 96 validated responses, of these 79% provided comprehensive input.
- Survey responses were geographically split: 47% Europe; 27% North America; 21% Asia (excluding Japan & China); 2% China; 2% Rest of World; and 1% Japan.

- Survey respondents were drawn from persons or groups interested in the gene editing, and undertaking or planning future investigation of CRISPR/Cas 9 in drug discovery related applications.
- Respondents came from 59 University/Research Inst./Gov't Lab/Not-For-Profit Facilities; 10 Biotech Companies; 8 Large Pharma; 8 Medical School/Hospital/Clinics; 5 Medium-Small Pharma; 3 Contract Research Organisations; and 3 Agrochemical/Agri-Biotech Companies.
- Most survey respondents had a senior job role or position which was in descending order: 22 principal investigators; 16 senior scientists/researchers; 12 post-docs; 10 professors/assistant professors; 9 research scientists/associates; 7 department heads; 6 section/group leaders; 5 directors; 5 graduate students; 1 research technician; 1 lab manager; 1 vice president; and 1 other.
- 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) Experienced User; 2) Limited Experience; 3) Applying To Drug Discovery Process; 4) Industry; and 5) University Research. The full report gives details of how these survey groups were segmented.
- <50% of respondents were currently undertaking CRISPR/Cas9 gene editing related to drug discovery applications, the remainder were not yet fully undertaking, but planned future investigation.
- Most respondents current experience of gene editing with CRISPR/Cas 9 technology and its potential in drug discovery was low i.e. have made some initial investigations.
- The median time period since respondents had first started using or investigating CRISPR/Cas 9 gene editing technology was 6-12 months ago.
- The median % use of CRISPR/Cas 9 gene editing technology in drug discovery was minimal use (<25% of research effort) today (2016).
- The median number of scientists working full time (FTEs) on CRISPR/Cas 9 gene editing in respondent's organisation today (2016) was 2 FTEs.
- Most respondents were applying or intending to apply CRISPR/Cas 9 gene editing to basic drug research.
- Oncology/cancer was the key disease or research area most targeted by CRISPR/Cas 9 gene editing.
- The majority want to achieve a gene knockout using CRISPR/Cas 9 gene editing technology.
- The majority were targeting their CRISPR/Cas 9 gene editing research against the human genome.
- Identification of new therapeutic targets was the main objective of CRISPR/Cas 9 gene editing in drug discovery.
- Most respondents had not investigated other gene editing technologies prior to CRISPR/Cas 9 availability.
- Efficiency (i.e. edit targets sequences at surprisingly high rates) was ranked the main advantage of CRISPR/Cas 9 gene editing technology.
- Complete genetic knockout, while minimising off-target effects was rated the potential benefit of CRISPR/Cas 9 in drug discovery most respondents were interested in exploiting.
- Allows for efficient introduction of engineered alterations into the genome was rated the most relevant features of CRISPR/Cas 9 technology to respondent's research.
- The bioinformatics tools most used to design sgRNAs were software supplied by vendors.
- The preferred method of delivery of CRISPR/Cas 9 components into the cell was standard chemical (lipidmediated) transfection.
- Gene knockout by non-homologous end joining (NHEJ) was the technology most used for CRISPR/Cas 9

experiments.

- The cell type most used today (2016) in CRISPR/Cas 9 research was tumor cell lines.
- A median of 26-50% efficiency of gene editing was typically achieved in CRISPR experiments.
- The downstream analytical technique most used to validate CRISPR/Cas 9 gene edits was PCR.
- The median number of guide RNAs (sgRNAs) designed per gene target edited was 2 sgRNAs.
- The majority were not undertaking multiplexing - but plan future investigation to deliver sgRNAs targeting multiple genes to multiplex CRISPR/Cas 9 gene edits.
- The majority plan to use CRISPR (guide RNA) libraries for target validation or drug mechanism studies, and prefer to use arrayed CRISPR libraries.
- A reporter assay was ranked the cellular response endpoints (outputs) of greatest value in the functional screening of CRISPR (guide RNA) libraries.
- A median of 2-3 CRISPR/Cas 9 gene editing projects were attempted per year today (2016).
- The median annual budget for CRISPR/Cas 9 gene editing related reagents was \$2.5K-\$5K per lab per year today (2016).
- A bottom-up model was developed to estimate the market for CRISPR/Cas 9 gene editing related reagents using respondent data on their budgets derived from this survey. The CRISPR/Cas 9 gene editing related reagents market (used specifically for drug discovery applications, as distinct from all other research applications) was estimated to be around \$30M today (2016). CAGR estimates and segmentation are given in the full report.
- The main components of the CRISPR/Cas 9 gene editing related reagents budget were individually purchased CRISPR/Cas9 reagents and transfection reagents.
- The supplier/vendor of CRISPR/Cas 9 gene editing related reagents that first comes into the mind of survey respondents was AddGene.
- The main suppliers from which respondents currently purchase the majority of their CRISPR/Cas 9 gene editing related reagents were AddGene, Millipore Sigma and Thermo Scientific.
- Delivery of CRISPR components into the target cell or organism was ranked what most limits work on CRISPR/Cas 9 gene editing today.
- All Respondents feedback on any unmet needs that exist today in CRISPR/Cas 9 gene editing related to drug discovery were documented.
- The full report provides the data, details of the breakdown of the responses for each question, its segmentation and the estimates for the future (2018). It also highlights some interesting differences between survey groups.

## Additional Details

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# Table Of Contents for CRISPR/Cas 9 Gene Editing In Drug Discovery Trends 2016 [Published by HTStec]

- Executive Summary . 2Table Of Contents 4Survey Methodology 5Organisation & Response Of Survey Participants 7Respondent's Company Or Organisational Origin . 8Respondent's Geographic Origin. 9Respondent's Job Role 10Respondents Currently Undertaking CRISPR/Cas9 Gene Editing In DD 11Current Experience Of Gene Editing With CRISPR/Cas 9 Technology 12Time Period Of Use Of CRISPR/Cas 9 Gene Editing Technology . 13% Use Of CRISPR/Cas 9 Gene Editing Technology In Drug Discovery. 14What Respondents Have Achieved Or Want To Achieve From Using CRISPR/Cas 9 Gene Editing Technology In Drug Discovery 15Number Of FTEs Working On CRISPR/Cas 9 Gene Editing 17Area In Drug Discovery Process Where Most Applying CRISPR/Cas 9 Gene Editing 18Key Diseases Or Research Area Using CRISPR/Cas 9 Gene Editing 19Summary Of Survey Findings (1) 20What Respondents Want To Achieve Using CRISPR/Cas 9 Gene Editing . 21Genomes Being Targeted With CRISPR/Cas 9 Gene Editing 22Main Objectives Of CRISPR/Cas 9 Gene Editing In Drug Discovery 23Other Gene Editing Technologies Investigated Prior To CRISPR/Cas 9 . 24The Advantages Of CRISPR/Cas 9 Gene Editing Technology . 25Most Wanted Potential Benefits Of CRISPR/Cas 9 In Drug Discovery (1). 26Most Wanted Potential Benefits Of CRISPR/Cas 9 In Drug Discovery (2). 27Most Relevant Features Of CRISPR/Cas 9 Gene Editing Technology (1) 28Most Relevant Features Of CRISPR/Cas 9 Gene Editing Technology (2) 29Bioinformatics Tools Used To Design Guide RNAs (sgRNA) 30Preferred Method Of Delivery Of CRISPR/Cas 9 Components Into Cells . 31Methodology Most Used For CRISPR/Cas 9 Experiments 32Use Of Different Cell Types In CRISPR/Cas 9 Research 33Efficiency Of Gene Editing Typically Achieved In CRISPR Experiments 34Downstream Analytical Techniques Used To Validate CRISPR/Cas 9 Gene Editing . 35Number Of Guide RNAs Designed Per Target Gene Edited . 36Multiplexing CRISPR/Cas 9 Gene Edits . 37Summary Of Survey Findings (2) 38Use Of CRISPR (Guide RNA) Libraries (1). 40Use Of CRISPR (Guide RNA) Libraries (2). 41Cellular Endpoints Of Greatest Value In The Functional Screening Of CRISPR Libraries. 42Number Of Different CRISPR/Cas 9 Gene Editing Projects Attempted Per Year . 43Annual Budgets For CRISPR/Cas 9 Gene Editing Related Reagents 44Market Estimate For CRISPR/Cas 9 Gene Editing Related Reagents . 45Breakdown Of CRISPR/Cas 9 Gene Editing Related Reagents Budget 46Breakdown Of CRISPR/Cas 9 Gene Editing Related Reagents Market Estimate 47Supplier Of CRISPR/Cas9 Gene Editing Related Reagents That First Comes To Mind (1) 48Supplier Of CRISPR/Cas9 Gene Editing Related Reagents That First Comes To Mind (2) 49Main Suppliers Of CRISPR/Cas9 Gene Editing Related Reagents (1) 50Main Suppliers Of CRISPR/Cas9 Gene Editing Related Reagents (2) 51What Most Limits Work On CRISPR/Cas 9 Gene Editing Today 52Unmet Needs In CRISPR/Cas 9 Gene Editing Related To Drug Discovery Today 53Summary Of Survey Findings (3) 54

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