Panorama Medicine
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Advanced drug discovery for diseases caused by transcriptomic errors

Panorama Medicine has built a combined genomics and advanced computational analysis platform to develop therapies for diseases treatable through transcriptome modulation. The company is looking to partner its therapeutic solutions through licensing options, or to collaborate on custom screening projects.

Panorama Medicine is an RNA genomics- and computing-powered drug discovery company that develops therapeutic interventions for a range of common and rare diseases caused by messenger RNA (mRNA) abnormalities. Splicing of pre-mRNA into mature mRNA is an essential step for gene expression in higher eukaryotes. Errors in this process generate defective mRNAs that can result in dysfunctional or missing proteins or proteins affecting cellular function and potentially causing disease. The company's proprietary transcriptome-wide drug screening platform Pan-ACEA (Panorama's Automated Compound Effect Analyzer) identifies drugs to treat diseases caused by RNA splicing errors or improper transcriptions.

"Panorama's technology comprehensively captures global transcriptomic responses to small-molecule compounds and matches the effects to Panorama's curated disease database," said Jae Lee, Interim CEO of Panorama. "This strategy provides alternative approaches to undruggable protein targets and efficiently identifies the compound's potential to treat numerous diseases."

Panorama offers opportunities for licensing partnerships to further develop therapeutic leads discovered through Pan-ACEA and for building collaborations around custom screens and compound evaluations.

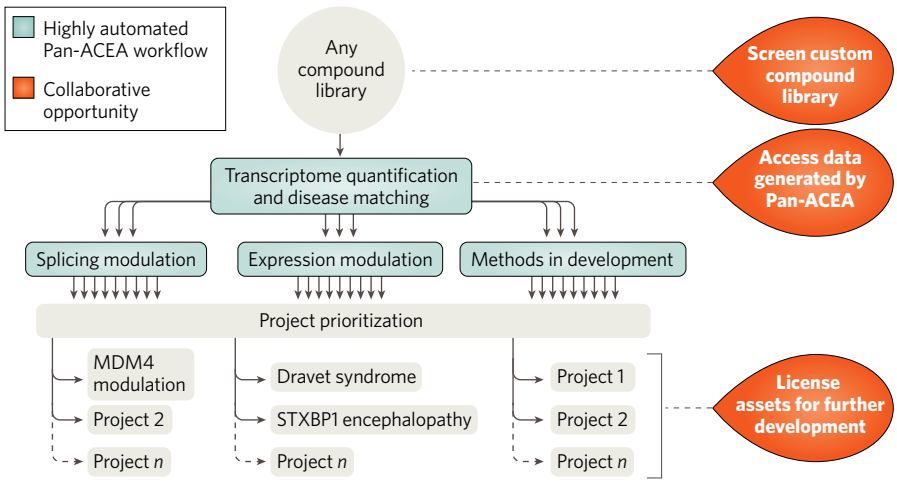

Fig. 1 | Panorama Medicine's Pan-ACEA platform. Panorama leverages genomics and computing to identify de-risked lead compounds that could help treat specific transcriptome-associated diseases.

Building projects through Pan-ACEA

Pan-ACEA leverages proprietary disease databases to identify lead compounds from a library of carefully selected compounds that induce changes in RNA splicing and/or expression (Fig. 1).

Pan-ACEA is able to detect all transcriptomic errors. Panorama has active projects which corrects aberrant splicing to restore proper balance of splice variants to treat diseases and increases expression of transcripts to treat diseases caused by insufficient transcript.

One mechanism for the inactivation of p53 in cancer is through the increased expression of full length MDM4. Several compounds were discovered through Pan-ACEA to modulate the splicing of MDM4 exon 6, resulting in reduced levels of full length MDM4 and subsequent p53 activation. The modulation of pre-mRNA splicing of MDM4 can be an attractive anti-neoplastic strategy to treat a subset of cancer patients.

Dravet Syndrome and STXBP1 encephalopathy with epilepsy are devastating childhood epilepsy syndromes caused by genetic mutations, resulting in the reduction of functional SCN1A and STXBP1, respectively. Currently, there are no effective treatment methods that can adequately control characteristic seizures in either of the epilepsy syndromes. Through Pan-ACEA, specific discovery compounds have been found to modulate SCN1A and STXBP1's expression, targeting the underlying genetic cause of the disorder.

According to Lee, "Pan-ACEA is a disease agnostic platform that is ideally suited to address any transcriptome-related disease rapidly and with high accuracy. This is of particular relevance when trying to advance first-in-class therapeutic options for numerous rare diseases."

Flexible partnering opportunities

Panorama has built a drug discovery platform that lends itself to a variety of partnership opportunities. The company's main thrust is in developing de-risked lead molecules primarily for rare, transcriptome-associated diseases. With a number of leads already in its pipeline for select diseases amenable to splicing modulation or modulation of gene expression, Panorama is the partner of choice for companies looking to in-license de-risked lead molecules for conditions triggered by aberrant RNA processing and expression.

Panorama is further seeking collaborations with potential partners interested in mining the company's unique and comprehensive transcriptome-associated disease database to access compound-, disease- or transcriptome-specific information. Such projects could lead to joint development programs and other collaborations.

Finally, Panorama also offers the possibility to interested parties to use its Pan-ACEA platform to screen alternative compound collections to identify other potentially addressable targets in transcriptome-associated diseases.

"Our platform provides us with great flexibility in terms of collaborating with interested external parties," said Lee. "While our primary goal is to develop new therapies in-house to advance the treatment of transcriptome-associated diseases, we are also eager to share the treasure trove of deep disease data and broad screening capabilities we have to complement external efforts leading to the same goal."

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