Panorama Medicine
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Advanced drug discovery for transcriptome-associated diseases

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 associated with 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 proteins or proteins that are missing altogether 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 such RNA splicing-associated diseases as well as other diseases treatable through modulation of the transcriptome.

"Panorama's technology comprehensively profiles global transcriptomic responses to small-molecule compounds and matches the effects to Panorama's curated disease database," said Mingfu Zhu, co-founder and CEO of Panorama. "This strategy 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.

Building a 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. This strategy allows Panorama to identify potentially de-risked compounds—all compounds in Panorama's library have been deemed safe in phase 2 studies but were terminated in later-phase studies owing to lack of efficacy for the original indications—that could treat specific transcriptome-associated diseases (Fig. 1). Pan-ACEA has the capacity to deliver drug candidates for multiple diseases simultaneously as well as to identify structurally diverse candidates for a particular condition at the same time.

Pan-ACEA can screen compounds for different kinds of transcriptomic errors, including aberrant splicing, which can be treated through modulating the balance between correct and aberrant mRNAs; insufficiently expressed transcripts, which can be treated through targeted modulation of expression; and some undisclosed directions that are under active development. Panorama has ongoing programs in all three modalities.

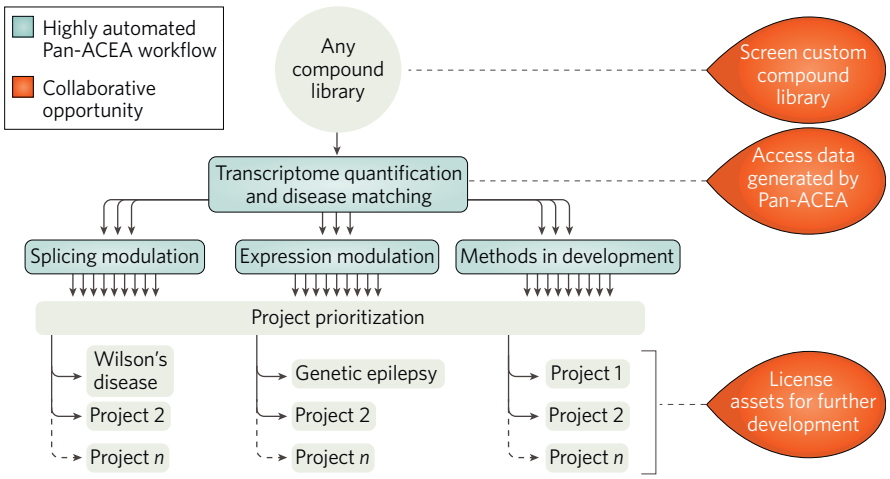

Fig. 1 | Panorama Medicine's Pan-ACEA platform. Panorama leverages genomics and computing to identify de-risked lead compounds that could help treat specific transcriptome-associated diseases.

Wilson's disease is a rare disease caused by aberrant splicing of the ATPase copper transporting β (*ATP7B*) mRNA, which results in accumulation of copper by the body and debilitating symptoms such as swelling, fatigue, abdominal pain, and uncontrolled or poorly coordinated movements. Panorama has identified a compound, Pano-002, that reduces exon skipping caused by a missense mutation and an intronic mutation associated with the disease.

Another lead program at Panorama focuses on a form of genetic epilepsy caused by reduced expression of a synaptic protein. The company has identified two compounds, Pano-013 and Pano-066, which promote increased expression of the gene.

According to Zhu, "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 partnering opportunities. The company's main thrust is in developing de-risked therapeutic leads 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 candidates 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 Zhu. "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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