PurMinds NeuroPharma Inc.
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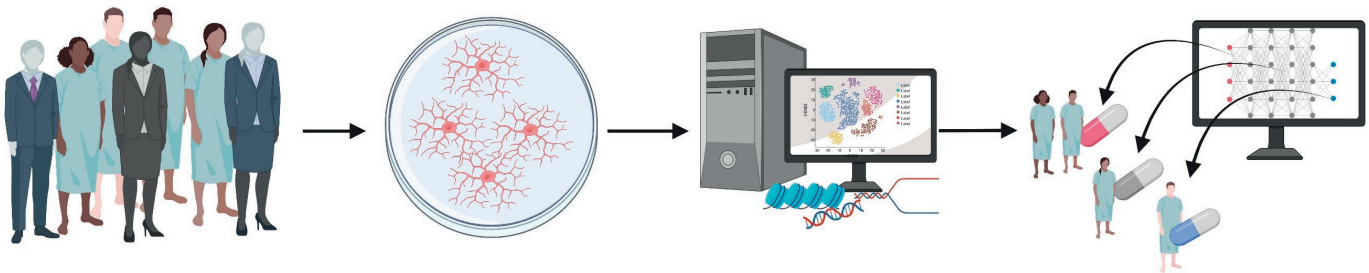
PURMINDS NEUROPHARMA

Developing precision neurotherapeutics takes patients

With a drug discovery platform built around patient-derived induced pluripotent stem cells and multi-omics, PurMinds NeuroPharma is de-risking the path to targeted brain therapeutics.



Leading-edge drug discovery and development process: the PurPrecision neuromedicine discovery platform.

The biopharmaceutical industry has a tried-and-true drug development method, moving from in vitro testing to animal models before taking the leap to clinical trials. The costs and fail rate are high, but the approach has produced many disease-modifying drugs. Just, not for neurological diseases.

The complexity of the human brain and central nervous system has left companies over-reliant on poor animal models that largely do not reflect human disease, which has stymied the development of therapeutics and led to widespread divestment by risk-averse big pharmaceutical players. That abdication has left openings for creative new approaches and led directly to the formation of PurMinds NeuroPharma. The company was launched by CEO Janet Qi in 2021 to find a better way to screen therapeutic leads and develop promising neurological disease assets, quickly coalescing around an approach championed by chief scientific officer, Jonathan Grima.

While a PhD student in neuroscience at Johns Hopkins University in Baltimore, United States, Grima and colleagues identified a novel therapeutic target for amyotrophic lateral sclerosis (ALS) and frontotemporal dementia (FTD). The researchers used induced pluripotent stem cell (iPSC)-derived neurons from patients with the most common genetic mutation of ALS and FTD to confirm a druggable connection to human neurodegeneration. This led to the development of BIIB100, which Biogen acquired from Karyopharm as a preclinical asset and sped into clinical testing for ALS. Grima and colleagues employed a similar approach to identify novel Huntington's and Alzheimer's disease targets. Grima was subsequently named to 'Forbes 30 under 30 in science' in 2019.

"Many cellular pathways that are disrupted in neurodegenerative diseases can be recapitulated using human iPSC-derived brain cells, which are generated from human blood or skin cells, but are effectively brain cells in a dish," said Grima. "This helps overcome one of the largest barriers in neurological drug development—the inability to biopsy and study brain tissue in live patients."

"The technology can potentially help predict efficacy of drugs in a cost-effective manner before initiating timely and expensive clinical trials. More importantly, our work may show that these iPSC-derived brain cells have more predictive validity and translational success than current animal models because this is a human model, but more studies are needed," said Grima. "We have to abandon this one-treatment-fits-all approach in neurology and psychiatry, where we've lacked the tools to connect the right drugs to the right patients. We're not only trying to improve the likelihood of clinical efficacy for our novel drug candidates, but hopefully identify biomarkers for populations most likely to respond, thus de-risking assets long before they reach human testing."

Diverse portfolio for neurodegenerative diseases

Today, PurMinds' portfolio includes small molecules and psychedelics. Its lead asset targets a specific post-translational modification that has been shown in preclinical testing to rescue nucleocytoplasmic transport defects, and prevent the formation of toxic protein aggregation that are believed to drive neurodegeneration across several diseases, including ALS and FTD. The company will soon perform therapeutic efficacy and IND-enabling toxicology studies for its two lead assets, and continue exploration of their therapeutic potential for other neurodegenerative diseases.

PurMinds' PurPrecision neuromedicine discovery platform will generate a large wealth of biological data from valuable human models and then mine this data using artificial intelligence and machine learning to uncover novel drug targets, dysfunctional molecular pathways, and biomarkers. This allows the company to screen for pathologic contributors to disease directly from the same patients whose biologic materials were used to generate these brain cells.

Data generated from the human models help PurMinds to continuously iterate and improve the platform, as well as guide clinical trial designs built on novel biomarkers for patient screening.

"Trial design has traditionally been a challenge in this space," said Albert Agro, PurMinds' chief medical officer. "Because neurodegeneration occurs over such a long period of time, and because symptoms alone don't stratify disease usefully, companies have been desperately seeking non-invasive biomarkers of disease. We can use the iPSCs to create genetic fingerprints for patients likely to benefit from targeted therapies, guiding patient selection and surrogate trial endpoints."

Partnering for the future

The company is continuing to explore partnerships and identify mature assets for in-licensing, while raising its series A to move the aforementioned assets into the clinic over the next year. The funding will also help progress the rest of its pipeline, which includes assets in lead optimization and a handful more of small molecules and psychedelics in development. "We're leading an ethos adjustment in neuropharma," said Qi. "We believe we have developed a unique and innovative discovery and development process that will hopefully translate to greater success for patients."

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