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DAx: exploratory disease research at Novartis " >

By matching technological advances and new scientific insights with unmet medical needs, we're finding new ways to help patients.

Therapeutic breakthroughs happen only at the convergence of scientific advancement and patient need, enabled by technological progress. Toward that end, the Exploratory Disease Area (DAx) group looks for opportunities to develop innovative treatments that improve the lives of people living with illnesses without a cure.

Induced pluripotent stem cell-derived liver cells shown with fluorescence microscopy. Courtesy of Christophe Antczak.

Led by Tewis Bouwmeester, the group deploys cutting-edge tools such as CRISPR, organs-on-a-chip and organoid models, genetics and computational methods to probe disease pathways and strategies for restoring the normal functioning of cells and tissues. Many of our researchers have backgrounds in pathway biology, stem cell biology and regenerative medicine. Together with disease experts, experienced drug hunters and clinical experts, our basic scientists work to identify areas of high unmet medical need and discover potential novel drug candidates for further evaluation.

We advance towards new frontiers in drug discovery with a focus on four therapeutic area priorities:

- Respiratory disease: Our team of respiratory researchers are using leading edge technology to understand respiratory diseases and fibrosis at the molecular level.
- **Liver disease**: Our researchers are evaluating therapeutic opportunities for patients with non-alcoholic steatohepatitis (NASH) and want to promote liver regeneration for other acute and chronic liver conditions.
- **Kidney disease**: We are using three-dimensional organoids and other tissue models to identify drugs that reverse autosomal dominant polycystic kidney disease. This genetic disorder often progresses to end-stage renal disease, which currently can only be treated with dialysis or transplantation.
- Benign blood disorders: We are using CRISPR in an effort to fix the genetic cause of sickle cell anemia. Our genome-editing and traditional low-molecular-weight compound approaches aim to restore normal hemoglobin levels in patient-derived hematopoietic stem cells that we re-infuse for treatment.

In ongoing research, we measured how vast networks of blood proteins undergo synchronized changes in patients with NASH or various chronic kidney diseases. This blood-based biomarker was developed using proteomics technology and provides a non-invasive tool for assessing disease progression and how patients respond to new drug candidates.

"We're always scouting for the next big opportunity," Bouwmeester says. "And in deciding which therapeutic areas to enter we ask: What is the unmet need? What is the current standard of care? Is there scientific reason to believe that we can improve on it in a transformative way? This is our fundamental approach."

Reimagine medicine with Novartis

Learn about opportunities to join our team

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