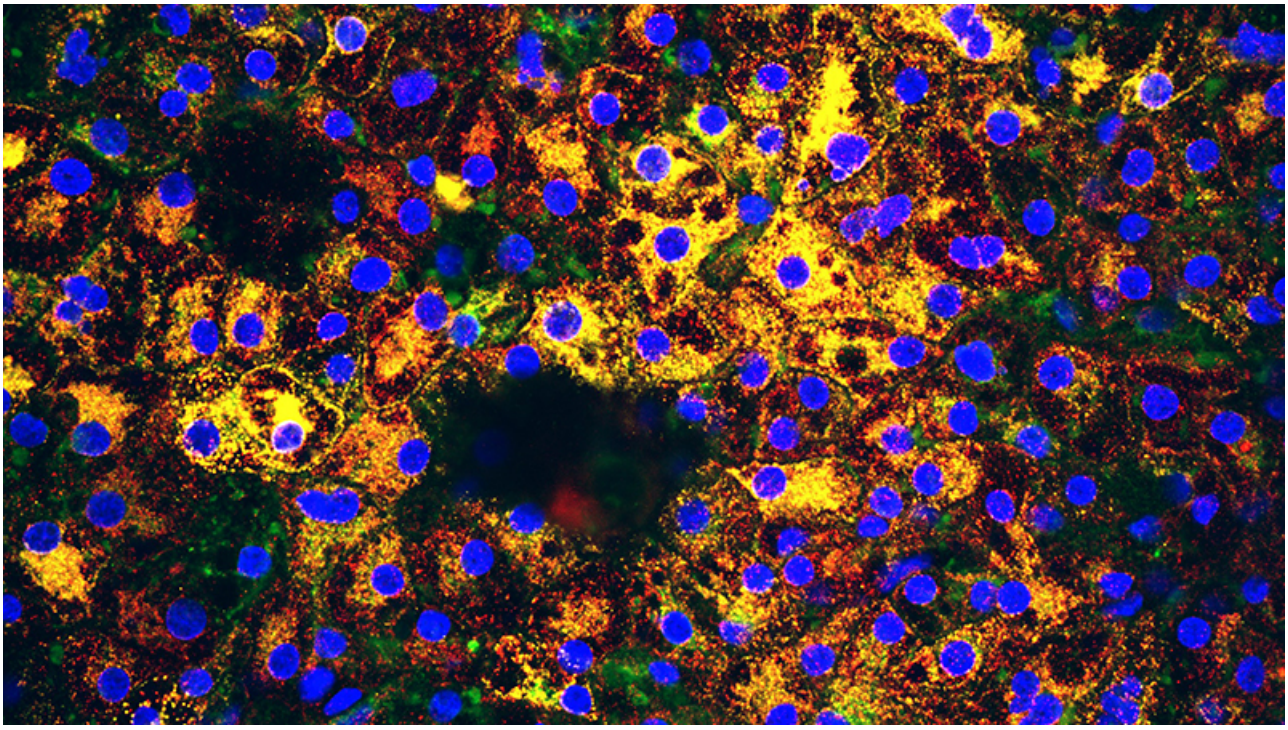
DAx: exploratory disease research at Novartis

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.

The group deploys cutting-edge tools such as CRISPR, organ-on-a-chip and organoid models, genetics and computational methods to probe disease pathways, and devises 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 two therapeutic area priorities and one multi-organ approach:

- **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.
- **Liver disease:** Our researchers are evaluating therapeutic opportunities for patients with acute liver failure and non-alcoholic steatohepatitis (NASH), and want to promote liver regeneration for other acute and chronic liver conditions.

- **Fibrosis:** We are also building a cross-organ research group to tackle the underlying mechanisms of fibrotic diseases. This new endeavor will focus on targets and pathways that are applicable across several systems with the expectation of developing therapeutics that address multiple fibrotic indications.

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.

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