

When bile acids tell a bigger story: how cholestatic biology is reshaping rare liver disease research

21.7.2026 - | Ipsen

The human cost of a system out of balance.

Over the years, I've seen firsthand how crushing fatigue and relentless itching can define daily life for so many people living with rare cholestatic liver diseases. It disrupts sleep, work and family life – all visible signs of a body struggling to maintain biological balance. These symptoms come from cholestasis, where bile flow from the liver is impaired, leading to a toxic accumulation of bile acids, heightening the severity of symptoms and driving progressive liver damage. But despite advances in our understanding, treatment options remain limited. It raises an important question: what if the key to new treatments lies in understanding the wider role bile acids play in the body?

Bile acids: the bigger story shaping the future of research

For a long time, bile acids were mainly discussed in terms of the damage their build-up can cause in the liver. But advances in cholestatic biology are revealing a much broader picture. Bile acids are now recognized as powerful signaling molecules that influence metabolism, inflammation and immune function throughout the body.

As this understanding has grown, so too has the way researchers approach rare cholestatic liver diseases. The focus is no longer only on impaired bile flow, but on the biological imbalances that drive disease progression. Across rare liver disease research, this shift is opening new avenues for treatment. Today, approaches such as IBAT inhibitors, PPAR agonists, FXR agonists and microbiome-modulating therapies are all being explored, reflecting a deeper understanding of bile acid biology.

What's particularly exciting is how these advances are beginning to reshape the way we think about care. By intervening earlier and targeting the drivers of disease, we can begin to move beyond symptom control alone. Biomarkers such as alkaline phosphatase (ALP) – a marker of cholestasis where normalization is tied to better long-term outcomes – are increasingly helping guide treatment decisions.

Ipsen: putting this science to work for patients

At Ipsen, our approach in rare liver disease is built on a deliberate shift: understanding bile acids as a complex, system-wide driver of disease and daily patient burden. This perspective defines how we accelerate our science – shaping the pathways we focus on and the clinical trials we design to deliver meaningful impact.

Across five rare cholestatic liver diseases, we are advancing a focused research strategy grounded in emerging insights in bile acid biology, applying this understanding to develop therapeutic approaches.

<https://www.ipсен.com/stories/science/when-bile-acids-tell-a-bigger-story-how-cholestatic-biology-is-reshaping-rare-liver-disease-research>