An interview with Prof. Xavier STÉPHENNE,

Head of Paediatric Gastroenterology and Hepatology at the Saint-Luc University Clinics

Could you tell us about your specialisation?

Trained as a paediatrician, I did my thesis in Prof. Etienne Sokal's laboratory on the quality of liver cells after cryopreservation and thawing. I then pursued a career as a clinician-researcher in translational and clinical research in paediatric gastroenterology and hepatology.

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Could you tell us about the expert centre you coordinate?

It is the Paediatric Hepatology Centre, which brings together a wide range of multidisciplinary expertise focused on rare metabolic diseases, including extrahepatic biliary atresia, which affects one in 20,000 births on average. This represents around ten new diagnoses per year in Belgium. In addition, these rare diseases are often genetic in origin, as is the case with progressive familial cholestasis. If the disease progresses to liver cirrhosis requiring a transplant, the expert centre calls on its medical-surgical unit, which performs around 30 transplants in children per year. In fact, this centre is the only one in Belgium to offer transplants to children of all ages.

What clinical trials are you currently participating in?

Conducted by the Paediatric Clinical Investigation Centre, these trials cover paediatric liver diseases as well as conditions affecting endocrinology, cardiology, nephrology and, of course, gastroenterology. Several ongoing gastroenterology studies are targeting new molecules (monoclonal antibodies) for the treatment of inflammation of the digestive tract. In addition, two studies on two inhibitors of bile acid absorption in the stomach have been granted marketing authorisation in Belgium for the treatment of Alagille syndrome. Phase IV (post-marketing) studies are ongoing. Other ongoing trials are looking at a new antiviral drug in liver transplant patients, diabetes and obesity in Belgian children, and familial hypercholesterolaemia: in this respect, interfering messenger RNA is raising high hopes for the treatment of rare diseases.

What do you see as the main challenges in meeting the medical needs of children with rare liver diseases?

It should be noted that paediatric hepatology is one of the subspecialties that are not recognised in Belgium. This poses a problem in cases involving multiple consultations, such as Alagille syndrome, a systemic disease that can affect several organs at once. Only a specialist can bill for the consultation and request reimbursement from the National Institute for Health and Disability Insurance (INAMI). This is all the more regrettable given that we have the only “full program” transplant centre in Belgium. Our recognition comes from our membership of two European Reference Networks (ERNs): RARE-LIVER and TransplantChild. In fact, the two inhibitors mentioned above can only be prescribed by a paediatrician who is a member of the RARE-LIVER ERN. These inhibitors are indeed in phases IV, which involve the acquisition of expertise that is continued in the post-marketing phase. When will we get a recognition by the Belgian authorities?

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