

Interview with Dr. Ravi Rao, Head of Research & Development, Chief Medical Officer at Sobi

RARE DISEASE WEEK 2021



About Ravi

Ravi Rao is Head of Research & Development and Chief Medical Officer of Sobi. He has joined Sobi in September 2020 from Aeglea Biotherapeutics where he had been the Chief Medical Officer. Ravi has long-standing experience in early and late stage development as well as medical affairs in immunology, specialty care and rare diseases spanning development, approval and launches of new drugs. Ravi was previously an academic rheumatologist at Imperial College and a post-doctoral fellow at Harvard University.

1) Can you please tell us a bit about Sobi?

Sobi is a global biopharmaceutical company dedicated to providing access to innovative treatments that transform the lives of people with rare diseases. With our head office in Stockholm, Sweden, the Sobi organisation spans more than 30 countries, delivering treatments to patients in over 70 countries across the globe. We focus our research mostly on the areas of Haematology and Immunology, and the overlap between them.

The area of haematology covers a wide range of diseases and problems, including those involving red and white blood cells, coagulation and platelets, and bone marrow. For example, thrombocytopenia is a condition characterised by abnormally low levels of platelets, also known as thrombocytes, in the blood. It comes in several forms, including an autoimmune form (ITP) and another connected to chronic liver disease (CLD) These are all rare conditions, which if left untreated, lead to higher rates of hospitalisation and potentially mortality.

The field of immunology has long been at the heart of what we do at Sobi, allowing us to gain extensive experience over many years. Our Immunology portfolio enables the treatment of serious, disabling or even life-threatening diseases such as haemophagocytic lymphohistiocytosis (HLH). Primary HLH is a rare, life-threatening disease with no approved treatment options outside the US. We urgently need new, more targeted medicines that will help babies to reach stem cell transplant, without causing them any additional suffering in the process.



2) What does Rare Disease Day mean for Sobi?

Rare Disease Day is a reminder to all of us of the challenges faced by the rare disease community. It reminds us of how much we still need to do to improve our knowledge of rare diseases and how more than ever we need to work together to raise awareness of rare diseases and improve treatment possibilities. At Sobi, we keep challenging the status quo and are dedicated to the continuous research and development of high-potential therapies that have a lasting impact on patients.

3) EURORDIS has recently published their final Rare2030 policy recommendations. What are Sobi's views of this work?

We share the view of EURORDIS and the Rare 2030 Panel of Experts that the role of the EU in health should strengthen: health issues know no border, especially rare disease challenges which cannot be properly addressed by a single nation alone. There is a need to streamline and ramp up public-private partnerships, especially at transnational level, for rare disease research. We agree that more concentrated efforts are needed to optimise the environment in which rare disease diagnostics, research, treatment and care take place. Ideally, we need an environment that fosters patient partnerships in all these activities.

4) In your opinion, what aspects need to change for Sobi to bring more innovation for people living with rare diseases?

As a rare disease company our efforts are focused on making sure that there are more treatments available for people living with rare diseases and that they can access our therapies. Any public policy reforms that encourage these efforts are extremely valuable such as regulatory incentives that help alleviate research uncertainties and long-term investment risks or greater convergence of HTA processes. In Europe, we must build on the past twenty years of scientific and regulatory developments and on the lessons from the COVID-19 pandemic to optimise and streamline regulatory pathways for orphan & paediatric drugs. We have reached a crossroad where we need visionary, long-term change if we want to make a positive impact on the future of the rare disease community. In many countries outside of Europe, there is still no formal or harmonized definition of rare disease which can serve as the basis for regulatory assessment of orphan medicinal products and access policy frameworks. Until such definitions and frameworks are established, the allocation of resources and health interventions for the rare disease community will be limited.

5) You mentioned earlier in the interview the importance of an environment that fosters patient partnerships. At the 2021 Black Pearl Awards the AKU Society received an award for Patient Centric Leadership and outstanding results from the DevelopAKUre Consortium. Can you please provide more details about this group and what it has achieved?

The DevelopAKUre programme was a series of major international clinical trials, run by a consortium of 12 European partners, including Sobi. This consortium researched and



developed a treatment against alkaptonuria, a rare disease with a prevalence estimated at around 1/250,000 to 1/1,000,000. Understanding the disease, conducting the trial, analysing and communicating the data, was possible thanks to great leadership and collaboration with patient organisations, academia, clinical research organisations and industry. This example demonstrates the value of multi-stakeholder collaboration to address the unmet medical needs of the rare disease patient community. Such partnerships should become the gold standard in health-related activities as also highlighted in the Rare 2030 Foresight study.

6) What are Sobi's plans in terms of research & development?

We currently have 11 programs which are focused on rare diseases in immunology and haematology as well as a number of diseases that span across these two areas. Each of our medicines is either a novel mechanism of action or brings the potential for significant improvement in current care. We are partnering and developing several medicines each of which may address a high unmet medical need in several diseases. In addition, we intend to broaden our geographic scope across several countries and now have R&D presence in Europe (Sweden and Switzerland), USA, China and Japan.

