GSK outlines approach to delivering advances in the treatment of Rare Diseases

Creating an environment that fosters innovation, intuition and scientific acumen to deliver breakthrough thinking and new medicines

GlaxoSmithKline (GSK) today provided further details about the strategic focus and development priorities of the company’s unit dedicated to rare diseases, which was launched in February 2010. Following the announcement today of a new alliance with Fondazione Telethon and Fondazione San Raffaele, Marc Dunoyer, Global Head of GlaxoSmithKline Rare Diseases, outlined his ambitions for the unit and its potential to deliver significant benefits to underserved patient groups worldwide.

“GSK has a well established history of successfully researching and developing orphan drugs to treat rare diseases. For companies like GSK to continue to make significant progress in developing new treatments to address rare diseases it requires unique skills, commitment and most importantly a deep understanding of the conditions. For example Atriance®/Arranon® (nelarabine), for a difficult to treat form of leukaemia that affects only a few hundred patients worldwide each year, was the result of the dedication of one scientist, Gertrude Elion, whose personal insights and dedication led to us understanding the potential of this molecule and how it could be used to help a small group of patients who were in urgent need of another treatment option.

“However, with less than 10% of patients with rare diseases currently being treated worldwide, we recognise the size of the challenge, but also the opportunity to deliver new medicines to patients.” He continued, “We believe the creation of GSK Rare Diseases will help us realise this opportunity. The unit provides us with the dedicated resources and focus that we believe is necessary to create the best possible environment to foster the innovation, intuition and scientific acumen needed to deliver the breakthrough thinking and create new medicines or approaches to tackling rare diseases.”

Dunoyer continued, “Our aim is to ensure that everyone within GSK Rare Diseases is fully immersed in the most up-to-date thinking. There is a very tight-knit community within rare diseases; there are small numbers of patients affected by these diseases; and they are very often experts on their condition. Treatment of patients with rare diseases is often undertaken by
very specialised physicians, who are also the clinical investigators seeking new treatments for these conditions.

“We believe the most effective way to become integrated into this community is to ensure that we have dedicated employees within GSK who can work with patients, physicians and academia to continuously extend our understanding.”

Disease prioritisation
With between 6,000 – 8,000 rare diseases, Dunoyer recognises that the unit’s efforts will need to be focused. “We will target those diseases where we realistically believe we can make a significant contribution; ideally we will be creating new medicines, but importantly by adding to the weight of scientific knowledge into rare diseases to the benefit of all working in this area.”

“Selecting the diseases to target won’t be easy and we will need to make difficult choices; we have adopted a systematic approach, which we believe will be critical to helping us make the right decision. Our aim is to have a list of around 200 priority diseases that the unit will target – this list will change over time as our scientific knowledge and understanding changes.

“Therefore it is critical that we are close to the rare diseases community and have the most up-to-date information about each disease, such as prevalence and severity, upon which to base our decisions. Other factors that will also influence our decision making process will include our belief about the potential for a treatment to help manage a disease, its complications, or even offer a cure. Not all rare diseases are ‘reversible’ or can be significantly improved by therapeutic interventions and it will be vital that we are science-led in making these distinctions.

He continued, “We will also incorporate our understanding of medicinal chemistry and biology to help ensure that we are selecting the right molecular targets or disease pathway. Again this will allow us to focus our R&D efforts in the areas where we have the highest probability of delivering medicines of value.”

“If we make the right decisions upfront, then ultimately the risk associated with discovery and development is generally lower as the conditions are often well defined and clinical development programmes tend to be small with robust endpoints. In most cases the molecular target is known, making it easier for treating physicians to diagnose patients, many of whom are excited to be involved in clinical development programmes that offer the promise of new treatments.”
“We believe that by applying this systematic rigour to our decision making process we will ensure the long-term success of the unit for patients, physicians and also as a sustainable business area.”

**Fuelling the R&D Engine**

“To select the right molecules requires a strong R&D engine to continuously generate a large candidate pool. This is where I believe GSK has a unique offering. The R&D pipeline for GSK Rare Diseases will be filled from within the unit, but also from across the entire GSK organisation. Every scientist, whatever therapy area they are working, has been tasked with considering how a molecule or platform technology being developed could be applied to treat one of the thousands of rare diseases.

“In practice this means that we will not be restricted to a small number of compounds that only have utility for a particular rare disease. We do not want to become a specialist in a single area of rare diseases, such as oncology, as we believe this will limit the potential of the unit. We want to create a diverse portfolio of compounds looking at a broad spectrum of rare diseases. I believe the strong GSK pipeline will give us enormous scope and access to novel compounds with mechanisms we understand that could be utilised to treat rare diseases.”

Moving on, Dunoyer also recognised the cutting-edge research being undertaken by external organisations. “We know there is a lot of exciting rare diseases work being undertaken at the moment, and we will be looking to augment our in-house capabilities by collaborating with organisations and institutions working in complementary areas to GSK. We have already formed a number of successful alliances, including Prosensa and JCR Pharmaceuticals, and we intend to build on these collaborations in our pursuit of the best science in this area.”

**Turning molecules into medicines**

“To make our molecules into medicines we will leverage GSK’s considerable expertise in areas such as formulation and manufacturing. One of the unique aspects for the unit within GSK will be the continuous involvement of GSK Rare Diseases from discovery to commercialisation. We know this is very important as it will help ensure that we remain close to both the treating physicians and importantly the patients.

“Critically I believe one of the most important capabilities of GSK, which is so vitally important in an area where there is a small number of patients spread world-wide, is our global reach – we believe we not only have the ability to discover and develop the medicines, but also to successfully deliver them to those patients who really need them. Never have I been more committed or excited about our potential to really make a difference.”
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