GENPHARM

Brining Cures to Rare Disease Patients in the MENA Region

300 million people live worldwide with a rare disease. Half of which are children suffering daily from devastating consequences of these rare diseases. More than 90% of the rare diseases are without an FDA approved treatment. Given these facts, a small dream was created 10 years ago hoping that one day we would be able to bring cures to MENA patients. This hope was called Genpharm.

More than 5 years ago, I was honored to join the unstoppable team at Genpharm in which our core value and what we lived for daily is to always put our patients first, deliver on our promises and to always be passionate about what we do. These values for me were the driving motive to take part in the patients' journey by securing them the niche products present worldwide for their rare condition.

We partner with different multinational companies across the globe each of them has the best-in-class treatment for a particular rare disease condition. We make sure to bring those treatments to the MENA region so that our patients can have a better and healthier lives through fast-track registration considering that these treatments are lifesaving orphan medications. Some examples of the disease areas we tackled are Duchene Muscular Dystrophy, Spinal Muscle Atrophy, Progressive familial intrahepatic cholestasis, Generalized Myasthenia Gravis, Mucopolysaccharidosis VII and many other rare diseases.

Leading the Regulatory Department, it is crucial to be able to provide access to MENA patients, especially children affected by rare diseases as soon as possible as this will affect the disease progression dramatically. We have managed to obtain technical approval through fast track for Translarna (Ataluren) in Saudi Arabia. Ataluren is a novel, orally administered small-molecule compound for the treatment of patients with genetic disorders due to a nonsense mutation. it is in clinical development for the treatment of Duchenne muscular dystrophy caused by a nonsense mutation (nmDMD). Ataluren is the first treatment approved for the underlying cause of DMD worldwide. We have also submitted regulatory files in multiple GCC countries to register 3 novel orphan drug products for the treatment of Duchenne Muscular Dystrophy. EXONDYS 51 is the first FDA-approved Duchenne muscular dystrophy treatment for patients who have a confirmed genetic mutation in the dystrophin gene that can be treated by skipping exon 51 and VYONDYS 53 which is an antisense oligonucleotide from Sarepta's phosphorodiamidate morpholino oligomer (PMO) platform, indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients with a confirmed mutation amenable to exon 53 skipping and finally, AMONDYS 45 which is an antisense oligonucleotide indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the DMD gene that is amenable to exon 45 skipping.

We have pioneered to bring the first gene therapy treatment 'Zolgensma' to the MENA region through Name Patient Supply to KSA, Kuwait, UAE and Qatar. Together, in collaboration with Novartis Gene Therapies (formerly known as AveXis Inc.) we have compiled the Regulatory dossier for registration through Verification Process in Kingdom of Saudi Arabia. Not only were we the first company to bring gene therapy to the MENA region but we were also the first to obtain approval to treat abroad eligible patients with the first therapy approved for eligible patients with early-onset MLD. This cross collaboration between Regulatory affairs and Market access team is one that I am particularly proud of as MLD is a very rare, fatal genetic disorder caused by mutations in the *ARSA* gene which lead to neurological damage and developmental regression. In its most severe and common forms, young children rapidly lose the ability to walk, talk and interact with the world around them, and most pass away before adolescence. Libmeldy is designed as a one-time therapy that aims to correct the underlying genetic cause of MLD, offering eligible young patients the potential for long-term positive effects on cognitive development and maintenance of motor function at ages at which untreated patients show severe motor and cognitive impairments so to have this level of care and treatment to eligible patients was nothing short of a true miracle.

Each day at Genpharm, we are motivated to pursue a better tomorrow alongside patients. We aim to do this through bringing cures and hope to our patients by continuing to register orphan drugs and gene therapy products in the region to ensure fast access to these niche products. For me this is what I live for, I live for making an impact in the healthcare sector. I live for making more parents feel secure about their children's health knowing that they can receive the best-in-class treatment in the world and go back home with a smile on their faces having a better hope for tomorrow.

We strongly believe that each patient has the right to the best care when treatments are available. Despite the many hurdles from regulatory to funding, our department's core mission is to never quit until the patients in need can access the right treatment in the fastest time possible.