THE GALIEN FOUNDATION

PRODUCT: **GIVLAARI®**

COMPANY: Alnylam Pharmaceuticals

THERAPEUTIC AREA & INDICATION:
Acute hepatic porphyria (AHP) linked to production of toxic porphyrin molecules in the bloodstream in adults

ABOUT THE PRODUCT

GIVLAARI is the first FDA-approved treatment for adults with acute hepatic porphyria (AHP)—a family of rare, genetic diseases characterized by potentially life-threatening attacks and, for some patients, chronic pain and other debilitating symptoms that negatively impact daily functioning and quality of life. The first-ever approved treatment utilizing Enhanced Stabilization Chemistry (ESC)-GalNAc-conjugate technology, and second-ever RNA interference (RNAi) therapeutic, GIVLAARI further validates Nobel-Prize winning RNAi science as a groundbreaking platform for the development of therapeutics for complex, serious conditions with limited treatment options.

THE GALIEN FOUNDATION

PRODUCT: **ONPATTRO®**

COMPANY: Alnylam Pharmaceuticals

THERAPEUTIC AREA & INDICATION:
Polyneuropathy caused by hATTR amyloidosis leading to loss of nerve and organ function

ABOUT THE PRODUCT

ONPATTRO is the first FDA-approved treatment for patients with polyneuropathy of hereditary TTR (hATTR) amyloidosis, a rare, debilitating and often fatal genetic disease characterized by protein buildup in peripheral nerves, the heart, and other organs. Based on Nobel-Prize winning RNA interference (RNAi) science, ONPATTRO, marked the arrival of a new class of medicines called RNAi therapeutics.

THE GALIEN FOUNDATION

PRODUCT: **Oxbryta®**

COMPANY: Global Blood Therapeutics, Inc.

THERAPEUTIC AREA & INDICATION:
Prevents inherited propensity for
the hemoglobin polymerization
that causes sickle cell disease

ABOUT THE PRODUCT

Oxbryta® (voxelotor) tablets is a hemoglobin S polymerization inhibitor indicated for the treatment of sickle cell disease (SCD) in adults and pediatric patients 12 years of age and older. This indication is approved under accelerated approval based on increase in hemoglobin. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trial(s). SCD is a rare genetic blood disorder that affects nearly 100,000 people in the United States, primarily of African descent, and millions worldwide. For more information, including Important Safety Information, please visit: https://oxbryta.com/pdf/prescribing-information.pdf.

THE GALIEN FOUNDATION

PRODUCT:

Zolgensma® (onasemnogene abeparvovec-xioi)

COMPANY: Novartis Gene Therapies

THERAPEUTIC AREA & INDICATION:

One-time gene replacement therapy for children under age 2 with spinal muscular atrophy

ABOUT THE PRODUCT

Novartis Gene Therapies imagined a world in which a gene therapy – with just one treatment – could address the root cause of spinal muscular atrophy. This devastating disease, caused by the lack of a functional survival motor neuron 1 gene, can result in irreversible loss of motor neurons that control breathing, swallowing, and basic movement. Today, we have delivered on this vision. Physicians have the option to treat children under two years old with a one-time, intravenous infusion called Zolgensma® (onasemnogene abeparvovec-xioi).reduce the annual rate of sickle cell pain crises to 1.63 vs 2.98 (equivalent to -45%) and the annual rate of days hospitalized to 4 vs 6.87 (-42%). The most common side effects (incidence ≥10%) include nausea, back pain, joint pain, and fever.

THE GALIEN FOUNDATION

PRODUCT: **GALAFOLD®**

COMPANY: Amicus Therapeutics, Inc.

THERAPEUTIC AREA & INDICATION:
Oral drug for treatment of Fabry Disease through repair of gene-induced enzyme deficiencies that lead to organ failures, stroke and early death in adults

ABOUT THE PRODUCT

Galafold® (migalastat) 123 mg capsules is an oral pharmacological chaperone of alpha-Galactosidase A (alpha-Gal A) for the treatment of Fabry disease in adults who have amenable GLA variants. In these patients, Galafold works by stabilizing the body's own dysfunctional enzyme so that it can clear the accumulation of disease substrate. Globally, Amicus Therapeutics estimates that approximately 35 to 50 percent of Fabry patients may have amenable GLA variants, though amenability rates within this range vary by geography. Galafold is approved in over 40 countries around the world, including the U.S., EU, U.K., Japan and others.