ZOLGENSMA
is a one-time infusion for patients with spinal muscular atrophy, with potential for long-term benefits. Zolgensma is one of the first drugs to use gene therapy as a form of treatment.

NKTR-181
is an industry-first analgesic designed to provide pain relief, but with less of the euphoric feeling attributed to other pain medications, thus reducing the likelihood of dependency.

GOLODIRSEN
is notable because it treats duchenne muscular dystrophy, a fatal disease with few treatment options.

UPADACITINIB
is a new treatment for patients with rheumatoid arthritis, in a market where several alternative treatments exist. However, Upadacitinib is notable because it has demonstrated superiority over Humira in trials.

VYNDAXEL
treats a rare condition called transthyretin amyloid cardiomyopathy (ATTR-CM), which can lead to heart failure if not properly treated. Vyndaqel is the first and only FDA-approved treatment for ATTR-CM.