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[redacted] *Significant advances in genetic research are helping to diagnose and treat many conditions for which patients previously had no therapeutic options available. Discovering which mutations lead to specific diseases and adapting the pathological molecular process may prove a turning point in the treatment of many genetic and rare diseases. The ability to target messenger ribonucleic acid (mRNA), specifically pre-mRNA, currently offers important new opportunities to treat some diseases using antisense oligonucleotides (ASOs). This technology could clearly impact health outcomes in other diseases in years to come and ultimately influence pricing, underwriting, and claims assessment. At present, there are several ASO treatments approved for use, and there are numerous ASOs in clinical trials for cancer, cardiovascular disease, metabolic, endocrine, neurological, neuromuscular, inflammatory, and infectious diseases. This article will look at some of the most significant factors in the development and use of ASOs and their potential impact on mortality and morbidity.*

quency of seizures and temporarily improved overall quality of life in the patient.

Perhaps the most success for ASOs has been in

which are caused by di f erent mutations, and hence

dystrophin-positive f bers in up to 23% of the patients treated with 30 mg/kg of eteplirsen.
(Vyondys 53) addresses a di f erent deletion (exon 53 skipping) in the dystrophin gene which a f ects about