

Abstract Title: An Ethical Case Study Concerning a Patient with Spinal Muscular Atrophy Enrolling in an Expanded Access Program

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We report the case of a 28-year old man with Spinal Muscular Atrophy (SMA) Type II (0 copies of SMN1 and 3 copies of SMN2). He reports worsening symptoms of weakness and muscle fatigue associated with his SMA diagnosis making him a candidate for a compassionate use drug program for Risdiplam run by Genentech. Risdiplam is an SMN modifying agent currently undergoing FDA review for treatment of SMA. Risdiplam would be the first orally administered SMA disease modifying agent if approved. The patient was accepted to be screened for this program. Prior to his screening visit, he experienced an acute medical problem which could potentially disqualify him. We will discuss the ethical issues regarding his participation in the program.