A Policy Analysis of Spinraza: An Orphan Drug for Spinal Muscular Atrophy

PURPOSE
An examination and evaluation of current policies of private, state, and federal insurance payers regarding approval of Spinraza for use in individual children will lead to recommendations for changes in local payment protocols and organizational involvement related to need for federal policies/regulations/guidelines for Spinraza/UODs for 5q-SMA.

BACKGROUND
The leading genetic cause of infant mortality is spinal muscular atrophy (SMA) which is linked to chromosome 5q. 5q-SMA is an autosomal recessive, progressive, neuromuscular disorder caused by a homozygous deletion or mutation or compound heterozygote of spinal motor neuron (SMN) 1, SMN2 copy number is important in determining the severity of the disease, which leads to profound physical disability, with intact intellectual capacity, and functioning. 5q-SMA affects approximately 1 to 2 per 100,000 persons worldwide. In 2017, the United States (U.S.) had an estimated 9,000 patients living with 5q-SMA. There are other forms of SMA, not caused by a mutation on chromosome 5q; these SMA variants are caused by defects in other genes on different chromosomes and are beyond the scope of this paper, which will concentrate on 5q-SMA, as Spinraza is limited to treatment of 5q-SMA.

METHODOLOGY
Collins (2005) 8-step health policy analysis tool for policy makers. Steps used are: 1) define the context, 2) state the problem, 3) search for the evidence, 4) consider different policy options, 5) project the outcomes, 6) apply the evaluative criteria, 7) weight the outcomes, and 8) make a decision.

RECOMMENDATIONS
1) Work with Congress to enact legislation, which will lead to FDA regulations requiring transparency of all pharmaceutical manufacturing

2) Work with committee to update 5q-SMA Standard of Care Guidelines

3) Work with committees on orphan drug pricing and standardization of prior authorization form

4) Employer support of practitioner active involvement (time away from clinical duties) in task force and committee work with professional organizations working on solutions to the issues surrounding ultra rare diseases