Biogen invites accredited members of the educational provider community to submit applications for independent, certified medical education grants subject to the terms described below. This request for education provides public notice of availability of funds to address areas related to the management of Spinal Muscular Atrophy patients.

**Background:**

Spinal Muscular Atrophy (SMA), an autosomal recessive neurodegenerative disorder which is characterized by motor neuron dysfunction, muscle weakness, and atrophy, is the most common cause of genetic death in infants¹. It is most commonly caused by a homozygous deletion or mutation in the survival motor neuron 1 (SMN1) gene on the long arm of chromosome 5, which results in deficiency of the survival motor neuron (SMN) protein. Phenotypic variability exists in SMA, primarily related to the variable number of copies of a paralogous gene, the survival motor neuron 2 (SMN2) gene, that an individual patient may possess. SMN2 contains a splice-site variant that leads to the exclusion of exon 7 from the mature mRNA transcript in the majority of cases, and therefore the production of a non-functional, truncated SMN protein. However, approximately 10% of the mRNA transcribed from each SMN2 copy is correctly spliced, allowing for the production of a functional SMN protein. Patients with a greater number of SMN2 copies generally develop milder disease phenotypes and the later onset of symptoms²-⁴.

For patients with neuromuscular disorders that are associated with deficits in mobility and weight-bearing, including SMA, there are associated risks of reduced bone mineral density, increased bony fractures, and the development of osteoporosis⁵-⁶. For some patients with SMA, particularly those who are non-ambulatory, severe contractures and scoliosis may also be manifestations of their disease which can contribute to significant morbidity and can influence clinical treatment and management decisions. According to the most recent update to the Standard of Care consensus guidelines published in 2018 addressing the rehabilitation and orthopedic needs of patients with SMA, there is growing evidence for the importance of regular physical therapy to promote motor functioning and improve the rates of progression of disease⁷. For the management of scoliosis in young children with SMA in whom pronounced growth is still anticipated, the 2018 consensus guidelines note that use of spinal orthoses is generally advocated as a palliative measure. Over time, however, with advancing curve magnitudes, rates of progression, and the development of adverse consequences of worsening spinal deformities such as respiratory compromise and functional impairments, spinal instrumentation may be required for...
these patients. Some practice variability exists even among experts when considering the most appropriate rehabilitative and surgical management approaches for patients, overall highlighting the importance of individualized care to account for their unique clinical characteristics.

Currently, the FDA-approved therapies for SMA are nusinersen, an SMN2 mRNA-directed antisense oligonucleotide, delivered intrathecally, for use in pediatric and adult patients with SMA; onasemnogene abeparvovec-xioi, which is a one-time intravenously administered adeno-associated virus 9 (AAV9)-mediated SMN1 gene replacement therapy, approved for children less than 2 years of age, and risdiplam, which is an orally administered small molecule that modifies SMN2 mRNA splicing, and is approved for patients 2 months of age and older.

**Educational Need and Professional Practice Gaps:**

The treatment landscape for SMA has changed substantially in recent years owing to the approval of 3 treatments which aim to increase the production of functional SMN protein. While the availability of therapeutic options has altered the trajectory of disease in SMA dramatically, biomechanical and orthopedic manifestations of disease still contribute to significant morbidity in many patients. The most recent Standard of Care consensus guidelines addressing the rehabilitation needs and orthopedic care of patients with SMA were published in 2018, and practice variability is known to still exist even among expert interventionalists, spine specialists, and other members of the extended multidisciplinary care team. A critical need therefore exists to educate clinicians regarding the evolving management considerations for the care of patients with SMA, including those with complex rehabilitation needs and orthopedic complications of their disease. Biogen seeks to address these important professional practice gaps through this medical education grant program.

**Educational Design and Focus:**

Biogen funding is intended to support multi-modal programs (i.e. with live/virtual and/or enduring components) which include a minimum of 3 activities that:

- Educate and increase disease awareness of SMA among members of the multidisciplinary care team involved in the management of SMA, with a focus on the orthopedic and biomechanical complications of disease, including overall bone health, scoliosis, and complex spine considerations
- Education on the current therapeutic options that exist in SMA, and the importance of providing individualized care to patients with SMA, tailored to their unique clinical needs
- Education on emerging research, best practice recommendations, and consensus guidelines for the multidisciplinary management of patients with SMA of varying genotypes and phenotypes across the lifespan

Grant proposals should include:

- **Needs Assessment/Gaps/Barriers:** Include a comprehensive needs assessment that is well referenced and demonstrates an understating of the specific gaps and barriers of the target audiences. The needs assessment must be independently developed and validated by the educational provider.
• **Target Audience and Audience Generation**: Describe methods for reaching the target audience(s) and any unique recruitment methods that will be utilized. The anticipated participant reach should be included, with a breakdown for each modality included in the proposal.

• **Learning Objectives**: Provide clearly defined and measurable learning objectives that address the identified gaps and barriers.

• **Educational Methods**: The ACCME calls for educational methods that are clearly designed to address the knowledge, competence and/or performance gaps that may underlie an identified healthcare gap. The proposal should demonstrate an understanding of instructional design issues as they relate to the gaps in the knowledge, competence, or performance of the targeted audience.

• **Program Evaluation and Outcomes**: Provide a description of the approach to evaluate the quality of the educational program. Additionally, describe the methods that will be used to determine the extent to which activity has served to close the identified healthcare gap. Programs should include an outcomes plan of at least Moore’s level 4.

• **Budget**: Include a detailed budget with rationale, including breakdown of costs for content per activity, out-of-pocket cost per activity and management cost per activity.

• **Accreditation**: Programs must be accredited by the appropriate accrediting bodies and fully compliant with all ACCME Criteria and Standards for Commercial Support℠.

• **Communication and Publication Plan**: Provide a description of how the provider will communicate the progress and outcomes of the educational program. Include a description of how the results of this educational intervention will be presented, published, or disseminated.

**Eligibility Criteria:**
Applicants must be in good standing and accredited to provide CME/CE by an official accrediting agency (e.g. ACCME, APA, ANCC, ACPE, etc.). The accrediting provider and, if applicable, the medical education provider (MEP) or other third-party vendors executing the activities are expected to comply with current ethical codes and regulations.

**Submission Instructions:**
If your organization wishes to submit an educational grant request, please visit [www.biogengrantsandgivingportal.com](http://www.biogengrantsandgivingportal.com) to access the online submission portal. Please include the RFE code (RFE-21-SMA-002) in the "Request for Education (RFE) ID Number" field in the online application.

**Grant Decision-Making Criteria:**
For information about the decision-making criteria, visit [https://grantsandgiving.biogen.com/](https://grantsandgiving.biogen.com/).

**Award Decision Date:**
Approvals and denials will be communicated via email notification no later than October 29, 2021

**Terms and Conditions:**
- All grant applications received in response to this RFE will be reviewed in accordance with all Biogen policies and policy guidelines. Please visit our website [https://grantsandgiving.biogen.com/](https://grantsandgiving.biogen.com/) for details on the grant structure, eligibility criteria, and requirements for participation.
This RFE does not commit Biogen to award a grant or pay any costs incurred in the preparation of a response to this request.

Biogen reserves the right to approve or deny any or all applications received as a result of this request or to cancel, in part or in its entirety, this RFE.

All communications about this RFE must be submitted through Biogen’s Grants & Giving Portal at www.biogengrantsandgivingportal.com. Failure to comply will automatically disqualify applicants.

References: