As part of our commitment to improving the lives of people living with rare diseases Alexion, AstraZeneca's Rare Disease supports quality, independent Continuing Medical Education (CME) designed to enhance patient care and health outcomes.

This call for grant applications provides public notice of availability of funds to address areas related to the multidisciplinary care of patients with NF1.

Deadline for Submission	November 30, 2023
Decision Notification	January 31, 2023
Primary Area of Focus	Rare Disease
Therapeutic Area	Neurofibromatosis Type 1 (NF1)
Geographic Focus	United States
CGA Code	AX001
Intended Audience	Adult and Pediatric Neuro-Oncologists, Heme-Oncologists, Neurologists, Neurosurgery, Clinical Geneticists
Budget	Up to $250,000
Educational Need	Neurofibromatosis type 1 (NF1) is a rare multisystem disorder that predisposes patients to tumor formation due to a deficiency in the neurofibromin protein.[1] Approximately 50% of cases are inherited.[2] NF1 is typically diagnosed in childhood but can be diagnosed throughout the lifespan. Approximately 30-50% of patients with NF1 develop plexiform neurofibromas (PNs) during their lifetime, which are tumors originating along peripheral nerves.[1,3] PNs often grow quickly during childhood and can cause disfigurement, pain, obstruction of vital structures, visual impairment, and bladder/bowel dysfunction.[3-5] PNs carry the risk of transformation into malignant peripheral nerve sheath tumors in 8-13% of patients, leading to severe pain and a poor prognosis.[6] Surgical resection of neurofibromas may be an option for patients; however, total resection of PNs is difficult due to their proximity to crucial structures and their high vascularity. Incomplete resection often leads to tumor regrowth following surgery.[7, 8] The range of possible manifestations of NF1 is broad, including ophthalmologic, cardiovascular, neurologic, cognitive, musculoskeletal, etc.[1] Due to the variety of clinical manifestations of NF1, both pediatric and adult patient care should be managed and directed by a multidisciplinary team (MDT) to facilitate the best clinical outcomes and patient/caregiver quality of life. In practice, the makeup of the MDT varies, but may include oncologists, neurologists, surgeons, geneticists, ophthalmologists, dermatologists, psychiatrists, etc.[9] There is a need for providers to understand the role of various specialties in the care of pediatric and adult patients with NF1 PN, and the value each specialty can bring to the management of patients throughout the treatment journey.

	Alexion, AstraZeneca Rare Disease seeks to support independent medical education designed to develop practitioners' understanding of: • The value of the MDT in pediatric and adult NF1 care • The importance of early engagement of the MDT to ensure comprehensive care of patients with NF1 and regular follow up to maintain appropriate treatment strategies • Appropriate monitoring recommendations for all members of the MDT at various stages of the patient journey, and for various manifestations of NF1 across the disease course, including brain tumors associated with NF1 • Key considerations and best practices for transitioning patients from pediatric care to adult care to ensure continued MDT engagement and support
Educational Design and Focus	Live event at relevant congress occurring in Spring/Summer 2024 (possibilities include, but are not limited to: ASPHO, AAN, ASCO, ISPNO, etc.) and an enduring online component following the congress event
Application Requirements	**Proposal must be independently developed and include the following:** • **Needs Assessment/Gaps/Barriers:** Include a comprehensive, well-referenced needs assessment that provides a detailed description of the educational / practice gaps and barriers of the target audiences. The needs assessment must be independently developed and validated by the educational provider. • **Audience Generation:** Describe methods for reaching the target audience(s) and any unique recruitment methods that will be utilized. • **Educational Strategy:** Provide clearly defined and measurable learning objectives that are clearly designed to address the identified gaps and barriers. 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 outcomes methodology that will be employed to measure the impact of the educational program and how these results will be presented, published, or disseminated. 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 and fully compliant with all ACCME Criteria and Standards for Commercial Support℠.

References

1. Blakeley JO et al. Neuro Oncol. 2016;18(5):624-638.
2. Pacot L, et al. Genes (Basel). 2019;10(9):633
3. Nguyen R, et al. *J Pediatr* 2011;159:652–655.e2
4. Dogra B, et al. *Indian Dermatol Online J*. 2013;4:195-198
5. Boyd K, et al. *J Am Acad Dermatol*. 2009;6:1-16
6. Evans DGR, et al. *J Med Genet.* 2002;39:311-314
7. Prada CE, et al. *J Pediatr.* 2012;160:461-46
8. Nguyen R, et al. *Genet Med*. 2013;15:691-69
9. Hirbe A, et al. *Lancet Neurol*. 2014;13:834-43

Program Requirements: The Program must be planned and executed as an accredited activity and fully compliant with the criteria and/or standards of commercial support for ACCME, AAFP, AOA, ACPE, ANCC, AANP, or NCCPA. Furthermore, the program will be educational and nonpromotional in nature and will be planned, designed and implemented in accordance with the U.S. Food and Drug Administration's Guidance on Industry-Supported Scientific and Educational Activities ("Policy Statement").

The Policy Statement and the ACCME Standards require, among other things, that (i) Institution conduct the Program independently and without control or influence by AstraZeneca over the Program's planning, content (including the selection of speakers or moderators), or execution; (ii) the Program be free of commercial bias for or against any product; (iii) Institution make meaningful disclosure of AstraZeneca support of the Program and any prior relationship between Institution and AstraZeneca, and the relationship, if any, between AstraZeneca and the speakers selected by Institution; and (iv) AstraZeneca not engage in, and Institution not permit any other sponsor to engage in, promotional activities in or near the Program room or advertise its products in any materials disseminated as part of the Program.

In addition, Institution is required by the Policy Statement and, if applicable, accreditation standards to ensure that any product discussions at the Program be accurate, objective, balanced and scientifically rigorous. This includes a balanced discussion of each product and of treatment alternatives, that limitations on data be disclosed, that unapproved uses be identified as such, and that for live presentations there be opportunities for questioning or debate.