

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 disease monitoring for Wilson Disease (WD).

<b>Deadline for Submission</b>	March 15, 2023
<b>Decision Notification</b>	April 15, 2023
<b>Primary Area of Focus</b>	Rare Disease
<b>Therapeutic Area</b>	Wilson Disease
<b>Geographic Focus</b>	United States
<b>CGA Code</b>	AX001
<b>Intended Audience</b>	Adult and Pediatric Gastroenterologists and Hepatologists
<b>Budget</b>	Up to \$250,000.00
<b>Educational Need</b>	<p>WD is a rare, inherited disorder of Cu metabolism that requires lifelong management.<sup>1,2</sup> If untreated, WD can be fatal, with most patients dying from liver failure or progressive neurologic disease.<sup>3,4</sup></p> <p>The clinical manifestations are predominantly hepatic, neurologic, and psychiatric, with many patients having a combination of symptoms.<sup>1,5</sup> Regardless of the initial presentation, patients often develop other systemic symptoms as the disease progresses.<sup>1</sup></p> <p>Long-term follow-up of patients with Wilson disease has shown that a proportion of patients deteriorate even when adequately managed with chelating agents, zinc, and/or liver transplant.<sup>6</sup> Poor treatment adherence has also been associated with neurologic and hepatic deterioration.<sup>2,4</sup> Some neurological symptoms of Wilson Disease have been shown to be refractory to treatment.<sup>7</sup> Thus monitoring for disease progression, which can enable early intervention and better outcomes, is strongly recommended.<sup>2</sup></p> <p>Given the importance of recognizing neurological and psychiatric symptoms early, Alexion, Astra Zeneca Rare Disease seeks to support independent medical education designed to develop practitioners' understanding of:</p> <ul style="list-style-type: none"> <li>• Burden of neurological and psychiatric aspects of the disease, in addition to the hepatic symptoms</li> <li>• Burden of treatment and necessity of treatment compliance to achieve copper balance</li> <li>• Appropriate laboratory and clinical assessments to monitor disease progression</li> </ul>

<sup>1</sup> European Association for Study of Liver. J Hepatol. 2012;56(3):671-685.

<sup>2</sup> Roberts EA, Schilsky ML. Hepatology. 2008;47(6):2089-2111.

<sup>3</sup> Patil M et al. J Clin Exp Hepatol 2013;3:321-336

<sup>4</sup> Roberts EA, Socha P. Handb Clin Neurol 2017;142:141-156

<sup>5</sup> Shribman et al. Ann Transl Med. 2019;7(Suppl 2):S60

<sup>6</sup> Merle U et al. Gut 2007;56:115-120

<sup>7</sup> Czlonkowska et al., Handb Clin Neurol 2017;142:101-119

<p><b>Educational Design and Focus</b></p>	<p>Multi-modal programs (i.e., with live/virtual and/or enduring components) including but not limited to:</p> <ul style="list-style-type: none"> <li>• Symposium at a national conference with a focus on educating healthcare professionals about liver or neurological diseases, examples include: <ul style="list-style-type: none"> <li>○ American Association for the Study of Liver Diseases (AASLD), November 2023</li> <li>○ American College of Gastroenterology (ACG), October 2023</li> <li>○ Digestive Disease Week (DDW), May 2023</li> </ul> <i>Slots should be secured by grant recipient.</i> </li> <li>• Interactive self-directed programs designed for impactful learner engagement using proven distribution channels</li> </ul> <p>Programs that:</p> <ul style="list-style-type: none"> <li>• Educate on the multisystemic symptomology of Wilson Disease</li> <li>• Educate on the approaches to monitor disease progression</li> <li>• Appreciate the value and importance of interdisciplinary collaboration in managing Wilson Disease</li> </ul>
<p><b>Application Requirements</b></p>	<p><b>Proposal must be independently developed and include the following:</b></p> <ul style="list-style-type: none"> <li>• <b>Needs Assessment/Gaps/Barriers:</b> 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.</li> <li>• <b>Audience Generation:</b> Describe methods for reaching the target audience(s) and any unique recruitment methods that will be utilized.</li> <li>• <b>Educational Strategy:</b> 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.</li> <li>• <b>Program Evaluation and Outcomes:</b> 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.</li> <li>• <b>Budget:</b> 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.</li> <li>• <b>Accreditation:</b> Programs must be accredited and fully compliant with all ACCME Criteria and Standards for Commercial Support<sup>SM</sup>.</li> </ul>

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## References

1. European Association for Study of Liver. *J Hepatol*. 2012;56(3):671-685.
2. Roberts EA, Schilsky ML. *Hepatology*. 2008;47(6):2089-2111.
3. Patil M et al. *J Clin Exp Hepatol* 2013;3:321-336
4. Roberts EA, Socha P. *Handb Clin Neurol* 2017;142:141-156
5. Shribman et al. *Ann Transl Med*. 2019;7(Suppl 2):S60
6. Merle U et al. *Gut* 2007;56:115-120
7. Czlonkowska et al., *Handb Clin Neurol* 2017;142:101-119

**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.