Early Access Programs at Astellas (as of 03-April-2023)

Before commercial availability, Astellas believes the safest and most effective way of providing access to our investigational therapies is through enrollment in our ongoing clinical trials. Astellas also recognizes the importance of utilizing the applicable regulatory and legal pathways for providing access to our investigational therapies when patients with life-threatening diseases have exhausted all available treatment options and are not eligible for clinical trials. For ongoing clinical trials sponsored by Astellas with investigational therapies currently in development, please visit https://clinicaltrials.gov/.

Astellas defines "early access" as the provision of an Astellas investigational therapy to patients outside of the clinical trial setting, upon receipt of an unsolicited request by a prescribing healthcare professional.[1] Where permitted by local regulations, Astellas will consider unsolicited early access requests from physicians for patients with no viable therapeutic alternatives. Requests are considered on a case by case basis, although provision of early access cannot be guaranteed when requested.

For certain Astellas investigational therapies, usually those targeting life-threatening diseases for which there are no currently available therapeutic alternatives, Astellas may initiate an early access program to manage individual requests more efficiently. An early access program may operate in a number of countries utilizing various legal pathways to access, depending on local laws and regulations. As such, program requirements may vary by country. Early access programs begin before marketing authorization, and typically end no later than commercial availability.

Below is a list of Astellas global early access programs which are currently operating or enrolling patients. These are programs that provide patients with access to investigational therapies that are currently in development and require physicians to follow an approved treatment protocol. A link will be provided for early access programs that are registered on ClinicalTrials.gov. Please follow the link to confirm if the program remains open to new patients.

Investigational Therapy	Program Status
Physicians seeking access to enfortumab vedotin	The enfortumab vedotin (ASP7465) early access program is open in certain countries where commercial access to enfortumab vedotin with reimbursement is not yet available. Early access to enfortumab vedotin is limited to patients who meet the eligibility criteria. Physicians may contact Astellas at EV.GlobalEAP.Request@astellas.com for additional information. Where approved for use, enfortumab vedotin is available under the brand name PADCEV®.
Physicians seeking access to enzalutamide	The global enzalutamide (MDV3100) early access program to treat eligible patients with castration resistant prostate cancer is closed to new patients. Where approved for use, enzalutamide is available under the brand name XTANDI®.
Physicians seeking access to gilteritinib	The gilteritinib (ASP2215) early access program to treat eligible adult patients who have relapsed or refractory acute myeloid leukemia (AML) with a FLT3 mutation is closed to new patients in all countries and regions. Where approved for use, gilteritinib is available under the brand name XOSPATA®.
Physicians seeking access to zolbetuximab	An early access program is not currently open and zolbetuximab is not available outside of clinical trials.

[1] This document aims to provide information on the ongoing Early Access Programs of the Global Astellas Group as a whole. The implementation status of each Early Access Program varies depending on the country or region. In Japan, Early Access Programs are conducted as Expanded Clinical Trials. Please visit PMDA's website at https://www.pmda.go.jp/review-services/trials/0019.html to see the ongoing Expanded Clinical Trials in Japan.

Investigational Gene Therapy

The Astellas Gene Therapy Center of Excellence is committed to developing and commercializing safe and effective gene therapy products for patients living with serious, rare neuromuscular diseases with limited or no treatment options. We appreciate the urgency of bringing these innovative products to patients, and this commitment drives our work each day.

The status of early access is provided below for later-stage gene therapies in development at Astellas.

For ongoing clinical trials sponsored by Astellas Gene Therapies, please visit https://clinicaltrials.gov/. Search on the medical condition and Astellas Gene Therapies.

Investigational Gene Therapy	Program Status
Physicians seeking access to resamirigene bilparvovec	Our priority is to demonstrate the safety and effectiveness of resamirigene bilparvovec (AT132), our gene therapy product for X-Linked Myotubular Myopathy (XLMTM), in order to obtain regulatory approval and make it available to appropriate patients as rapidly as possible. The safety of patients, healthcare professionals, and our employees is of utmost importance to us. To that end, we reviewed our current early access policy and maintain that it would not be appropriate to provide early access outside of a clinical trial at this time. Our decision on early access was not taken lightly at the time we implemented it, and the same is true now, with many complex issues being considered. It remains critical that we continue to focus on completion of the clinical trial for XLMTM to determine potential safety and efficacy for the broader XLMTM patient population.
Physicians seeking access to other investigational gene therapies in development	At this time, participation in clinical trials is the only way for patients to gain access to investigational gene therapies that are currently in development. For physicians or healthcare providers interested in additional information, please contact AGT_medinfo@astellas.com.