Expanded Access to Investigational Medicines

Purpose and Scope

Neurocrine Biosciences is a leading neuroscience-focused, biopharmaceutical company with a simple purpose: to relieve suffering for people with great needs but few options.

Clinical trials are an essential part of the development and registration of innovative medicines, and critical to making those innovative medicines available to underserved patients, families, and caregivers. In the normal course of researching, developing, and marketing medicines, Neurocrine Biosciences works with Healthcare Professionals (HCPs), patients, and healthy volunteers to conduct clinical trials evaluating the safety and efficacy of investigational medicines prior to pursuing regulatory approval. While administration within the framework of controlled clinical research is optimal, there may be occasions when it is in the interest of some patients to have access to investigational medicines when enrollment in a clinical trial is not possible. This Policy provides general guidelines for Neurocrine Biosciences' consideration of requests for access to its investigational medicines outside of clinical trials ("expanded access").

Expanded Access

When an individual is ineligible to participate in a clinical trial, or there are no available studies, depending on local regulations there may be other opportunities for expanded access to investigational medicines. These mechanisms for expanded access may have different names or approaches, such as Named Patient Program or Named Patient Use, Compassionate Use, Early Access or others, depending on the geography and what may be allowed by the respective regulatory authorities.

Where individuals have a serious or life-threatening disease or condition for which Neurocrine Biosciences has a potential therapy and where all alternative treatment options, including enrolling in available clinical trials are not suitable, expanded access may be a suitable option. Neurocrine Biosciences will carefully review all expanded access requests submitted by a physician or qualified HCP, on a case-by-case basis.

Criteria Considered for An Expanded Access Request

Neurocrine Biosciences considers the following criteria when reviewing requests for expanded access:

- An independent, unsolicited request has been received from the treating physician or qualified HCP.
- The individual has a serious or life-threatening disease or condition that Neurocrine Biosciences is investigating, and no comparable or satisfactory alternative therapy is available to treat the disease or condition.

- The individual does not qualify to participate in any ongoing clinical trial in a reasonably accessible geographical location (as determined by Neurocrine Biosciences).

- A Neurocrine Biosciences investigational medicine with sufficient clinical safety and efficacy data in humans with the disease is available and these data indicate potential benefit of expanded access of the investigational medicine.

- There is adequate supply of the investigational medicine to support expanded access treatment.

- Provision of expanded access will not otherwise jeopardize, or introduce undue, negative risk, to an ongoing, investigational program.

- Expanded access is permitted under local laws and regulations.

The physician or qualified HCP providing medical care to the individual must:

- Voluntarily initiate the request for expanded access. Neurocrine Biosciences does not solicit requests for expanded access.

- Supervise administration of the investigational medicine in alignment with any expanded access framework requirements (e.g. cohort protocol) established by Neurocrine Biosciences.

- Obtain informed consent from the individual patient and/or the patient's legal guardian(s) for treatment use of the investigational medicine, and collection of patient data as appropriate.

- Carry out the protocol for treatment use, in countries where this applies, under appropriate regulatory and ethical standards, including regulatory agency and/or Institutional Review Board (IRB) or Institutional Ethics Committee (IEC) review, as applicable.

- Maintain and release to regulatory agencies, and to Neurocrine Biosciences, all treatment records and data as specified.

- Maintain facilities for the safe and secure receipt of medicines in adherence with relevant legislation and guidance.

- Understand and commit to taking on the responsibility of prescribing an unlicensed medicine.

- Support the collection and reporting of safety data on the medicine as required by applicable regulations.

Making a Request for Expanded Access

Requests for expanded access to a Neurocrine Biosciences investigational medicine must be made by the patient's treating physician or qualified HCP. Physicians or qualified HCPs may submit a request by emailing the following information to medinfo@neurocrine.com :

- Date of request.
- Requesting HCP's name, contact information, address (including country), and professional designation (e.g., MD, DO, NP) or additional qualifications.
- Name of investigational medicine being requested along with HCP's intended treatment plan, including therapeutic indication and expected duration of treatment.
- Medical rationale for supporting favorable risk/benefit to patient including relevant patient information and clinical details, availability of therapeutic alternatives, and patient ineligibility for available clinical trials.

All information submitted as part of a request will be maintained in the strictest of confidence, adhering to all applicable patient data privacy regulations, and used solely for the purpose of evaluating a patient's eligibility for expanded access.

Confirmation of receipt of written requests will be provided within 3 working days. Each request will be reviewed fairly and promptly by a cross-functional team including qualified Neurocrine Biosciences experts. A written or oral response will typically be provided to the requesting HCP withing 15 working days with either an application decision or request for additional information. Neurocrine Biosciences is unable to commit to supporting all requests for expanded access. Requests for expanded access may not be made directly by an individual patient or a patient's parent/legal guardian or caregiver.

Additional information regarding the portfolio of Neurocrine Biosciences clinical development programs may be found on the Our Pipeline page and details regarding ongoing Neurocrine Biosciences clinical trials may be found at www.clinicaltrials.gov .