Santhera Pharmaceuticals USA's position on offering compassionate use to investigational medicines

At Santhera Pharmaceuticals USA, our mission is focused on developing innovative medicines for rare and other diseases with high unmet medical need. For investigational drugs, consistent with our mission at Santhera Pharmaceuticals USA, we encourage awareness and consideration of participation in clinical trials.

Investigational drugs are drugs that are not approved by regulatory agencies like the United States Food and Drug Administration (FDA). Clinical trials are used to test investigational drugs to establish their safety and efficacy before they are approved. Approval by regulatory authorities like the FDA is the only way to make treatments broadly available to the patient population at large.

During clinical development, Santhera Pharmaceuticals USA believes that participation in clinical trials is the preferred way for patients to access an investigational drug because these trials generate the efficacy and safety data needed to determine whether the investigational drug should be approved. Additional information about Santhera's clinical trials of its investigational products can be found at: clinicaltrials.gov.

However, when it is not possible for a patient to participate in a clinical trial and all other available treatment options have been exhausted, the patient's physician may seek special access to one of Santhera's investigational drugs through compassionate use, which may also be called expanded access or pre-approval access.

When designing a formal expanded access program (EAP) or any other compassionate use program, Santhera consults with physicians, patient groups and medical experts to understand the unmet need and to help determine the eligibility criteria.

When evaluating requests for compassionate use of an investigational drug, Santhera Pharmaceuticals USA considers *all* of the following criteria:

1. The disease or condition being studied is serious or life-threatening.
2. There are no comparable or satisfactory alternative therapies or clinical trials available.
3. Sufficient preliminary efficacy and safety data exist to support an assessment that the benefit for the patient outweighs the potential risks and that the potential risks are not unreasonable in the context of the disease or condition being treated.
4. Sufficient clinical data is available to identify an appropriate dose of the investigational drug.
5. There is adequate drug supply to support the ongoing and necessary clinical trials as well as to support approved compassionate use in a sustainable and equitable manner.
6. The patient is not eligible to participate in any ongoing clinical trials of the investigational drug.
7. Compassionate use will not adversely affect the clinical development program, in particular, the initiation, conduct, or completion of the clinical trials that are required for regulatory approval.
8. The unsolicited request is made by a qualified and licensed physician. The requesting physician must have the expertise and facilities appropriate for the administration of the investigational drug and for monitoring, managing, and reporting any side effects and the patient's overall experience.
9. All required regulatory and institutional approvals have been obtained. The patient must provide written informed consent.

We encourage patients to speak first with their treating physician about their eligibility to enroll in a clinical trial. If a treating physician believes that expanded access may be the only option for a patient, the physician should contact Santhera Pharmaceuticals USA to make a formal request on behalf of a patient by sending an email to: EAP.US@santhera.com

Requests for compassionate use will be individually reviewed in accordance with these criteria. Santhera Pharmaceuticals USA is committed to evaluating all requests for expanded access in a fair and equitable manner. All requests will be evaluated by medical professionals and decisions will be based on available scientific evidence at the time of the request.

Questions regarding Santhera Pharmaceuticals USA's ongoing EAP program(s) or policy can be forwarded to: EAP.US@santhera.com

Additional information regarding expanded access for individual Santhera products can be found on clinicaltrials.gov using the following hyperlink(s) and ClinicalTrials.gov identifier(s):

NCT03433807
Expanded Access Protocol (EAP) of Idebenone in Patients With Duchenne Muscular Dystrophy
https://clinicaltrials.gov/ct2/show/NCT03433807?term=NCT03433807&rank=1