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1.0 PURPOSE

- 1.1 This policy describes the principles and analysis that Catalyst Pharmaceuticals, Inc. (Catalyst) will use to determine whether to grant requests for Compassionate Use Access to the products it is developing.

2.0 SCOPE

- 2.1 This policy applies to all requests for Compassionate Use Access for Catalyst products that are in development, i.e., not approved for use, in the territory or jurisdiction requested.

3.0 RESPONSIBILITY

- 3.1 All assessments and determinations required in the policy prior to Compassionate Use Access are done either directly or under the direction of the Catalyst Chief Medical Officer (CMO) through his/her designees. Any assessments or determinations made by designees of the Catalyst CMO must be approved by the Catalyst CMO.
- 3.2 All Catalyst employees are responsible for understanding that any requests for Compassionate Use Access, or for information about the process, need to be directed to the Catalyst CMO, or his/her designees.

4.0 REFERENCES

- 4.1 21 CFR Part 312, Subpart I

5.0 DEFINITIONS AND ABBREVIATIONS

- 5.1 **Compassionate Use Access:** When access to an investigational medical product (i.e., one that has not been approved by FDA or relevant regulatory agency) is given to a patient who has no other treatment options outside of a clinical trial.
- 5.2 **Expanded Access Program:** A larger scale program, usually directed by the manufacturer, that provides Compassionate Use Access to a number of patients under a treatment protocol or a specific treatment IND.
- 5.3 **IND:** An abbreviation for Investigative New Drug Application which is a filing made with the US FDA to allow for investigative use, such as a clinical

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trial, of a drug for an indication for which it has not been approved.

6.0 ATTACHMENT(S)

N/A

7.0 POLICY

7.1 Receiving Requests for Compassionate Use Access

7.1.1 Formal requests for Compassionate Use Access to Catalyst products in development may only be made by licensed prescribers with authority to administer and oversee treatment of the patient(s) on whose behalf the request is/are made.

7.1.2 All such requests that come to Catalyst need to be directed to the Catalyst CMO or his/her designee.

7.1.3 The Catalyst CMO, or his/her designee, must document all formal requests received. Such documentation should include the requestor, the diagnosis of the patient and other details necessary for the Catalyst CMO to complete the two-step analysis described in Sections 7.2 and 7.3.

7.1.4 All such records should be kept for at least three (3) years and until the product is approved or development discontinued, whichever is later.

7.2 Determining Whether a Product is Appropriate for Compassionate Use Access

7.2.1 The Catalyst CMO, or his/her designee, must review the available information about the product to determine whether it is appropriate for Compassionate Use Access. Such information includes, but is not limited to:

7.2.1.1 Whether there is sufficient available evidence to support a conclusion that the product is likely to be both sufficient safe and efficacious for the indication to benefit a patient;

7.2.1.2 Whether it has been established that access on a compassionate use basis will not compromise any planned or ongoing clinical trials or the overall regulatory approval pathway for the product;

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- 7.2.1.3 Whether there is adequate supply of the experimental therapy available for a sufficiently foreseeable need to treat the patient(s);
- 7.2.1.4 Whether the compound can both be administered and is it logistically feasible to make it available outside of the clinical trial setting; and
- 7.2.1.5 Whether any other complications exist which would make the product inappropriate for Compassionate Use Access.

7.2.2 The Catalyst CMO, or his/her designee, should document the overall conclusion as to whether or not the product is appropriate for Compassionate Use Access based on all the available information. Any decision by a designee should be reviewed and ratified by the Catalyst CMO. This conclusion should be reached and documented within 30 calendar days, whenever possible.

7.2.3 If the conclusion is that a product is not appropriate, then the requestor needs to be informed of this and a record of that communication maintained.

7.2.4 Once a product is determined to be appropriate for Compassionate Use Access, the Catalyst CMO, or his/her designee, can rely on that rationale for further requests as long as all subsequently approved requests are reviewed to ensure that the rationale is appropriate based on the factors noted above.

7.2.5 If a requestor is not satisfied with the Catalyst CMO's decision and wishes to submit additional information or rationale as to the appropriateness of the product for Compassionate Use Access, then the requestor may do so and the Catalyst CMO should revisit the decision, again within 30 calendar days of receipt of the request to review. Additionally, a product that at one point is determined to not be appropriate for Compassionate Use Access may subsequently be determined to be appropriate based on subsequent information or intervening developments.

7.3 **Determining Whether a Patient is an Appropriate Recipient for Compassionate Use Access**

7.3.1 Once a product has determined to be appropriate for consideration of Compassionate Use Access, the Catalyst CMO, or his/her designee, then must review the specifics of the patient(s) for whom Compassionate Use Access is requested. To determine whether a

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patient is appropriate, the Catalyst CMO, or his/her designee, should determine:

- 7.3.1.1 That relevant underlying medical conditions of the patient(s) do not pose safety risks that have not been sufficiently studied;
- 7.3.1.2 The request has been made by a licensed prescriber with expertise and facilities appropriate for the administration of the therapy, monitoring, managing and reporting side effects;
- 7.3.1.3 The condition of the patient(s) is/are of a type and stage commensurate with the indication for which the investigational therapy is likely to be approved;
- 7.3.1.4 The patient(s) does/do not qualify for participation in ongoing or pending clinical trials; and all necessary regulatory/institutional approvals have been obtained to allow the administration;
- 7.3.1.5 Whether any other conditions or situations exist that indicate that the patient's participation in Compassionate Use Access is not beneficial to the patient or product.

7.3.2 Once the Catalyst CMO, or his/her designee, has determined whether or not the patient(s) is/are an appropriate recipient of the product through Compassionate Use Access, the decision should be documented, and the records kept with the original request and the requestor should be notified of the determination.

7.3.3 A requestor who wishes the Catalyst CMO's decision to be revisited, should submit any additional information regarding the patient that might affect the Catalyst CMO's decision relative to the criteria in Section 7.3.1.

7.4 **Expanded Access Programs and Treatment Protocols**

7.4.1 Either on its own accord or at the request of a regulatory agency, Catalyst may choose to create an Expanded Access Program for a particular product. In the United States, this would be done by submitting a treatment protocol to the IND or by submitting a separate Treatment IND.

7.4.1.1 Once the document is accepted by the regulatory agency, the Expanded Access Program will be initiated

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and conducted per the protocol under the supervision of Catalyst’s Clinical Operations and within Catalyst’s policies and procedures for clinical trials.

8.0 CHANGE HISTORY

Version	CC#	Change Description
01	NA	Original
02	See Document Signature Page	Renumbered from P60-001 to P60-002 to correct inadvertent duplicate policy numbers. Also, reviewed as part of periodic review for updates/revisions and no other changes needed.