

## **Comments on proposed draft of “National Policy for Treatment of Rare Diseases”, formulated by Ministry of Health and Family Welfare, Government of India**

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We are a non-profit patient-oriented organization focussing on a rare kind of muscular dystrophy called GNE Myopathy. However, we are also working for the benefit of the rare disease community in general in India. As such, we are very impressed with the Ministry of Health and Family Welfare’s initiative in drafting a National Policy for Treatment of Rare Diseases (the “policy”). It is critical for India to create an appropriate policy framework for tackling rare diseases and this is a timely exercise in that direction. We hope that legal and enforcement mechanisms will also be enacted to give teeth to the policy.

While the policy is commendable in many aspects, we are writing to express some of our concerns with certain aspects of the policy. We have divided our comments into categories, which are stated below. We sincerely hope that you will take these comments into consideration before the final policy is created.

### General comments:

We feel that the policy does not adequately represent the interests of the rare disease community at large and focuses only on certain diseases in a manner that may prove to be disadvantageous to the larger rare disease community in the long term. The focus of the current policy is to fund ERT of a few lysosomal storage disorders (LSDs). It talks about creating a corpus fund that will help defray the cost of the enzymes for a few patients. A different approach needs to be taken to address problems faced by a majority of the rare disease community, making up millions of patients in India.

The primary focus of a rare disease policy should be on developing and funding cheaper and better treatments/cures of rare diseases. For example, large scale funding in gene therapy can help us to acquire path-breaking technology potentially providing a complete cure for a large number of diseases, which no longer require patients taking expensive treatments throughout their lives. India is particularly advantageously placed to make a global impact through the development of these therapies with some support from the government. This will help to solve macro-economic allocative inefficiency as well as reducing life time treatment costs of LSDs. Therefore, we believe that the policy should shift its focus to the development of treatments for rare diseases in order to address the most critical but neglected issue in rare diseases.

The policy does not reflect on a number of issues as detailed below.

1. The policy needs to provide more guidance on diagnosis and management of different types of rare diseases within the country. This is a critical issue as wide-scale diagnosis is required for any epidemiological studies. The existing accredited centres are only a handful and also do not cater to different types of diseases. Since diagnosis also requires expensive, technologically intensive DNA analysis, the policy should consider utilising both public and private resources.
2. The current definition of a rare disease should be considered as an interim definition and used only for initiating activities under the policy. The policy should have provision for periodically reviewing definitions. We think that the numbers of rare disease patients will increase once proper mechanisms for diagnosis are in place.
3. There is very little emphasis on “Research & Development” in the draft policy. More cost effective treatments developed in India for a wide range of rare diseases will create greater economic viability and efficiency and will have greater societal benefits. The policy should dedicate funding for not only developing cures in India, but to also develop inexpensive assistive devices to make life comfortable for Indian patients. In fact, the policy should place more emphasis on “assistive devices” since these are needed by many patients to carry out their daily activities and to be less dependant on others.
4. No concrete measures have been proposed or any policy guidance suggested for an Orphan Drug Act. This is undoubtedly a necessary part of any regulatory framework on rare diseases and is missing in the policy.
5. The policy does not mention anything by way of establishing international collaborations with governments and other organisations globally. Given the challenges faced in the development of treatments for rare diseases, the fastest and most cost effective mechanisms for treating rare diseases involve global collaborations in areas of science, technology and medicine. Further, incentivising international entities to conduct clinical trials in India for rare diseases, supporting Indians to participate in global clinical trials and easing regulatory approvals for companies marketing orphan drugs will pave the way for many beneficial developments in rare diseases.
6. Many recommendations of the policy are based on current state of our knowledge and technical advances on the horizon have not been considered. For example, categorisation of diseases into three categories is arbitrary and does not reflect current effort or future advances (Table 2). With reference to Table 2, it may be possible that through the advancement of technology category 2 and 3 diseases may become category 1 diseases. The possible outcomes that can be brought about through therapies that are on the horizon need to be taken into account.
7. We believe that emphasis only on importing ERTs for a few LSDs has made this policy skewed with a narrow vision to help only a small group of patients. There are a number of other equally efficacious drugs for other rare diseases for which similar import restrictions should be eased. In addition, import restrictions and duties on assistive devices should also be substantially eased.

#### Specific Comments:

Specifically we would like to point out the following issues with respect to the draft policy.

1. Section 4.2 A

The policy proposes a number of committees at both the Central and State level, though it is not clear what will be their terms of references. It appears that the major objectives of these committees will be to disburse corpus funds for available treatment. We think that the institutional framework suggested by the policy will create more complexity and confusion and suggest that there should be one high level committee that will look into all aspects of rare diseases, including R&D and regulatory approvals.

2. Section 4.2 B

The major priority of the policy should be two points that have been mentioned. These should be taken under independent action points

“Taking measures to improve research.....orphan drugs etc.”

-Taking measures, legislative and otherwise.....more affordable”

-Last point should be modified to add “and other orphan drugs” after ERT.

3. Section 4.3.1 Point (g)

It needs further clarity. Our priority should be accurate and fast diagnosis and development of cures for these diseases. For these we need a highly supportive policy that encourages accelerated approvals. A special policy and legal framework must be put in place which takes into account concerns of the patients and helps to harness global effort for rare disease drug development. The policy should have provision for use of experimental drugs on “compassionate use” where there is no therapy available. The framework should incorporate rules that will allow marketing and import of drugs for rare diseases in India.

4. Section 4.3.1 Point (k-4)

Part of the corpus should be used for funding to develop new therapies, particularly clinical trials of promising drugs as these can significantly reduce cost of the Government and the society by making therapies available for more diseases at lower prices than ERTs.

5. Section 4.3.1 (l)

It is not clear how a poor family will access the portal. Primary health centres should be trained to participate in this process.

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