

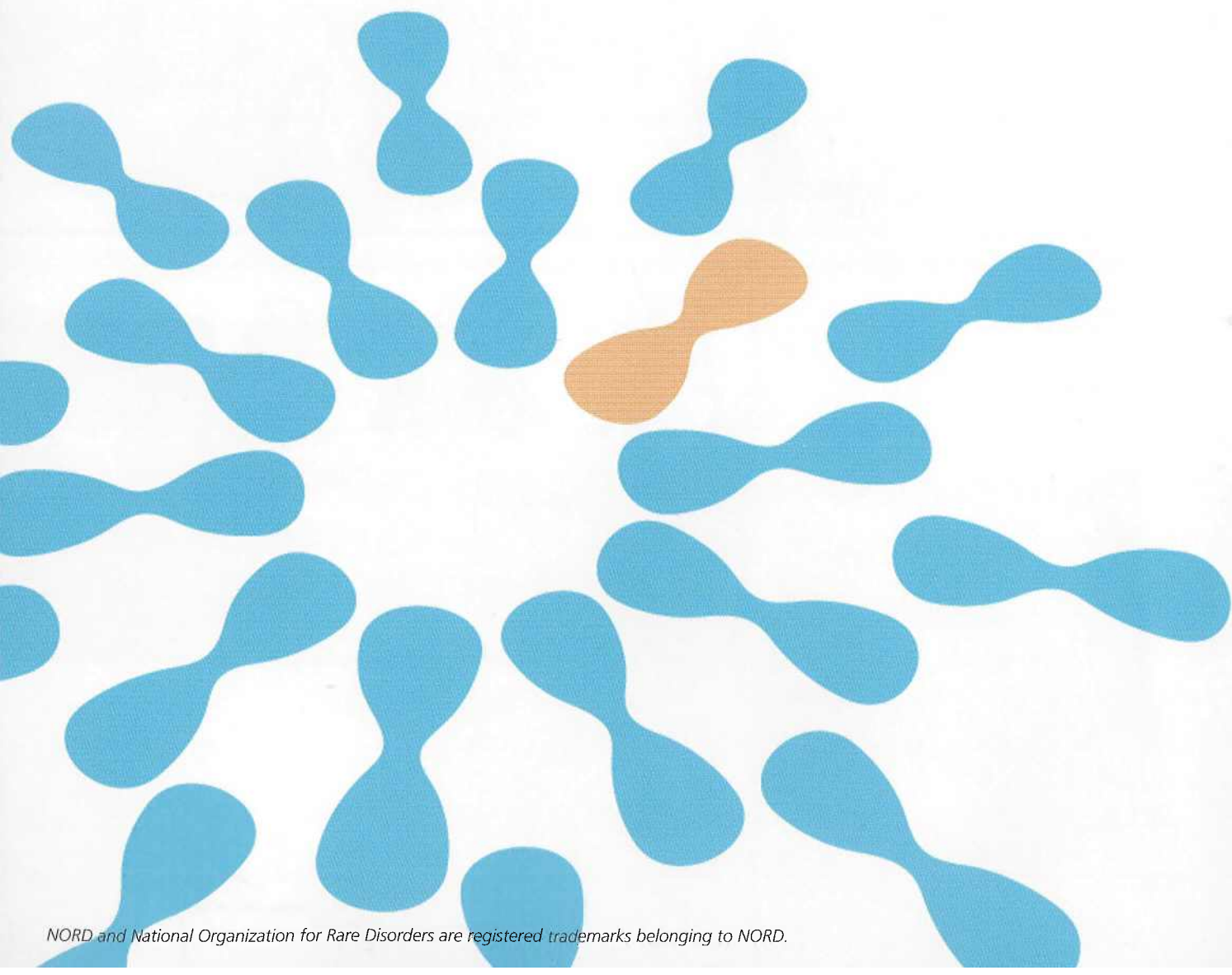
# National Organization for Rare Disorders

Partners  
IN  
PROGRESS  
2009

## A Summary of the Partners in Progress Summit

May 14, 2009 • Washington, D.C.







**The healthcare system we know today is going to be completely transformed over the next 12 months. Now is the time for action.**



**— Tommy Thompson, J.D.**

## **Background**

The National Organization for Rare Disorders (NORD), which advocates policies in support of the nearly 30 million Americans with rare diseases, sponsored the, "NORD Partners in Progress Summit," on May 14, 2009, in Washington, D.C. to establish its policy agenda.

NORD gathered together 21 national thought leaders on a Blue Ribbon Commission to discuss how to address the dual challenges of 1) stimulating innovation in the development of new therapies from their discovery through the regulatory review process at FDA, and 2) ensuring that those patients have full access to proper diagnosis and treatments.

## **Innovation**

Because a rare disease might affect only a few hundred or a few thousand people, and can therefore easily be ignored or misunderstood, innovation in developing new treatments must be supported from discovery through the Food and Drug Administration (FDA) approval process. NORD's traditional position has been that national policies, including regulatory and reimbursement policies, must take into account the special needs of patients with rare diseases and must be sufficient to protect their health and well-being without being so burdensome that they limit the development of therapies.

The importance of an environment that encourages and supports the study and research of rare disorders and the therapies used to treat rare diseases cannot be overstated. Indeed, it is through the study of rare diseases that the scientific and medical communities come to better understand and define how physiological processes operate in the vast majority of persons. Moreover, there is a dearth of dedicated government research funding for most rare diseases.

## **Access**

Patients with rare diseases must have full access to proper diagnosis and affordable treatments. Many rare diseases are poorly understood, under-diagnosed, and/or have no approved treatments. Indeed, of the approximately 7,000 rare diseases that have been identified thus far, fewer than 200 have an FDA-approved treatment. The objective of the Summit was to elucidate policies that would improve the timely diagnosis of individuals with a rare disorder, as well as policies to ensure full and timely access to therapies that are available.

Given this backdrop, the Summit aimed to achieve a clear and concrete policy agenda for patients with rare diseases, and in particular, to gain an understanding of:

- Federal policies that especially affect patients with rare diseases – the opportunities they offer, the obstacles they create; and ways to improve these policies; and
- Policy changes that NORD should advocate to assure innovation and access to therapies for rare diseases.

## The NORD Agenda that Emerged

Based on the presentations and discussion at the day-long Summit, the following ten goals emerged as being key elements in NORD's policy agenda:

**GOAL #1** – NORD should expand its current role to serve as a catalyst for public-private collaboration (government, industry, academia, and patient groups) to create an environment for the development of new treatments for rare diseases. In this effort, NORD should serve as the non-profit, neutral body representing the patient community in FDA's Critical Path Initiative.

**GOAL #2** – NORD should work with FDA to establish greater certainty in the orphan product approval process, in particular with respect to clinical trial design and endpoints. For example, NORD should seek to develop valid natural histories for rare diseases that can be used to define clinical endpoints. What is needed is a new paradigm for orphan drug development. Greater certainty would encourage investment in, and the development of, products for rare diseases.

**GOAL #3** – NORD should identify needed changes in the FDA law, regulations, and policies to encourage and facilitate product approvals. FDA is the gatekeeper for new products, but beyond that also sets the standard for clinical trials and product development. NORD should advocate for FDA to have the tools and policies needed to support orphan product development.

**GOAL #4** – NORD should seek to obtain greater funding for FDA and the National Institutes of Health (NIH), and should continue to urge the Social Security Administration (SSA) to expand its Compassionate Allowance program. These agencies serve central roles in supporting innovation and access for patients with rare diseases. Their ability to continue to foster programs to benefit patients with rare diseases largely is a function of their budgets and the support they receive from the rare disease community.

**GOAL #5** – NORD should develop systems that will enable greater patient access and participation in clinical trials of rare diseases. Recruitment to clinical trials for orphan drugs and medical devices often is an obstacle to timely clinical development.

**GOAL #6** – NORD should seek to assure that there are no lifetime limits on medical care. Most rare diseases are chronic and, therefore, lifetime limits on expenditures for ongoing treatment affect patients with rare diseases. As the government and private insurance companies become even more focused on cost savings, the special needs of patients with rare diseases must not be ignored.

**GOAL #7** – NORD should seek to assure reimbursement for off-label uses of drugs used to treat patients with rare diseases. The vast majority of patients with rare diseases have no FDA-approved medicines. When treatments are used, they often are medicines that are approved to treat common diseases but not rare diseases. Physicians are legally able to prescribe drugs for any patient who may, in their professional judgment, benefit from them, but reimbursement policies increasingly are denying payment for uses not specifically approved by the FDA. This means that patients with rare diseases may be denied reimbursement even when the accepted standard of care is to use a product that is not approved by the FDA for that specific use.

