

March 17, 2021

The Honorable Frank Pallone  
Chairman House Energy and Commerce  
United States House of Representatives  
Washington, D.C. 20515

The Honorable Cathy McMorris Rodgers  
Ranking Member House Energy and Commerce  
United States House of Representatives  
Washington, D.C. 20515

Dear Chairman Pallone and Ranking Member Rodgers,

On behalf of National Marrow Donor Program® (NMDP)/Be The Match®, thank you for the opportunity to provide our input on today's hearing on "The Future of Medicine: Legislation to Encourage Innovation and Improve Oversight."

For over three decades, through a competitively bid contract with the Health Resources and Services Administration, NMDP has been entrusted to operate the federally authorized bone marrow program that matches living unrelated adult donors with patients in need of a life-saving blood cell transplant, designated by Congress as part of the C.W. Bill Young Cell Transplantation Program (Program).

Today, patients in need of a blood cell transplant, without a suitable match within their family, have access to more than 39 million potential donors making a cure a reality for thousands of Americans each year. To date, NMDP has facilitated more than 111,000 transplants and partners with over 190 hospital transplant programs in facilitating transplants.

As trusted leaders in advancing treatments for those facing life-threatening blood cancers and other blood diseases, we provide ground-breaking research, innovative technologies, patient support, and patient education that saves lives. Therefore, we are uniquely positioned to comment on the impacts of this agenda that aims to revolutionize how the United States provides care to patients and finds new and better treatments for diseases.

In honor of our longstanding public-private partnership with several United States government agencies, transplant centers across the country, and academic institutions, NMDP is grateful for the opportunity to provide input on the legislation before you today.

We write in support of the following policies before you:

- **Improving Access to Clinical Trials**
- **Innovations in Telehealth Delivery Beyond the Pandemic**
- **Accelerating Research & Creation of ARPA-H**
- **Pandemic Preparedness Rare Disease Support Program**

#### **Improving Access to Clinical Trials**

The goal of improving diversity in clinical trials is aligned with NMDP's vision to democratize cell therapy and advance equal outcomes for all. Clinical trials are a key step in advancing potential new treatments for patients with blood cancers and blood diseases, but these trials are not accessed equally by all patients.

NMDP supports the effort to improve access to clinical trials by covering the cost to travel for clinical trials through NMDP's Jason Carter Clinical Trials Program, which helps patients and families find and join clinical trials.

NMDP supports CURES 2.0's efforts to improve clinicaltrials.gov. NMDP offers a searchable database through the Jason Carter Clinical Trials Search and Support Program, one-on-one support from clinical trial navigators, as well as helpful clinical trial information and resources, such as financial assistance and educational fact sheets. An improved clinicaltrials.gov enhances our ability to provide the most accurate and up-to-date information to the patients we serve.

Additionally, the Blood and Marrow Transplant Clinical Trials Network (BMT CTN) was created in partnership with the NHLBI, NIK, and NIC because of a critical need for multi-institutional clinical trials focused directly on improving survival for patients undergoing hematopoietic cell transplantation. Since 2001, the BMT CTN has opened more than 30 multi-institutional Phase II and III trials, involved more than 100 transplant centers, and enrolled thousands of patients.

NMDP will continue to work to remove barriers to clinical trial participation for patients with transplant-treatable blood disorders and cancers, and supports H.R. 6000 – CURES 2.0, H.R. 5030 – DIVERSE Trials Act and H.R. 6584 DEPICT Act in their intent to do the same.

#### **Innovations in Telehealth Delivery Beyond the Pandemic**

The COVID-19 public health emergency (PHE) has highlighted the need for and importance of telehealth in enabling physicians to care for their patients.

Telehealth services implemented during the PHE greatly improved and expanded access to our patients, whose immune systems are ablated in treatment, by allowing those patients to avoid exposure to infectious pathogens during hospital and clinic visits, when clinically appropriate. Telehealth has also helped improve patient outcomes by making follow-up appointments safer and easier.

We ask that you make permanent these changes to Medicare and Medicaid originating site requirements. A durable telehealth extension will continue to ensure that both patients and their donors can access care when they need it as it takes a community to deliver cures for bone marrow, blood stem cell, and cord blood transplant recipients.

#### **Accelerating Research & Creation of ARPA-H**

The public-private collaboration between academic centers and industry, facilitated by the NMDP and CIBMTR, has resulted in improvements in patient outcomes following blood cell transplantation. Such collaborative research includes developing new cell therapies directed at eradicating malignant disease, preventing infection, and treating graft-versus-host disease, the top three causes of patient demise following transplantation.

As leaders in innovative cell therapy, we are pleased to share our support for today's legislation accelerating research:

- **Grants for Novel Trial Designs and Other Innovations in Drug Development:** This type of trial design can be particularly useful in the evaluation of complications following complex medical procedures such as blood stem cell transplant and/or immunotherapy for cancer where

prevention of relapse and/or graft versus host disease is critical for addressing common causes of treatment failure.

- **Increasing Real World Evidence:** While randomized clinical trials remain the gold standard for evaluation of efficacy and safety of novel therapies about the standard of care, real-world evidence captured at the population level is critical for continued surveillance of efficacy and safety of new treatments. This is especially relevant to populations that are typically underrepresented in many clinical trials. Real-world evidence data collection strategies that mandate reporting, like the HRSA Stem Cell Therapeutic Outcomes Database, ensure that nearly all patients are represented providing insights into groups that may be less likely to participate in clinical trials and research in general. NMDP encourage the uniform capture and coding of health information, as it would greatly enhance our ability to leverage real-world evidence.
- **Incentives for Decentralized Trials:** Decentralized trials can increase access to clinical trial care and improve patient outcomes. By allowing for virtual care visits, patients will not need to travel to the center, may not need to take as much time off work, and can lessen the burden for patient caregivers. Trials using telemedicine or other digital technologies have the greatest potential to affect underserved patients.
- **ARPA-H:** NMDP supports the creation of ARPA-H to pursue high-risk/high-reward breakthrough innovations. As leaders in cellular therapy, we have a major role in demonstrating that blood cell transplantation from related and unrelated donors can be used to successfully treat over 70 blood cancers and blood diseases and supports similar efforts to deliver seminal treatments to patients in need of a life-saving cure.
- **Research Investment to Spark the Economy:** NMDP support efforts to study the pandemic, to support blood cancer and blood disease patients in the current pandemic and to prepare for future pandemics. The CIBMTR and BMT CTN are conducting a prospective observational study on the efficacy of SARS-CoV-2 vaccines in transplant recipients and cellular therapy recipients. The CIBMTR is also capturing data on COVID-19 infections and outcomes in the same population. Lessons learned from both will continue to serve the community in the ongoing management of COVID in the setting of transplant and cellular therapy and provide insights into the effective management of immunocompromised patients in future pandemics.

### **Pandemic Preparedness Rare Disease Support Program**

Nearly all of the diseases and disorders that can be treated by blood stem cell transplant qualify as rare diseases. We support policies and programs that support our patients in future pandemics.

NMDP/Be The Match supports the implementation of the policies before you today not only to significantly advance medical innovation, but also to make accessible these innovative therapies to patients regardless of race, place, or circumstance.

Please do not hesitate to contact me with any questions, and thank you in advance for your consideration,



Brian Lindberg  
**National Marrow Donor Program**  
Chief Policy Officer, Chief Legal Officer and General Counsel