



Delegate Sickles, Chair
House Appropriations Committee
Health and Human Resources Subcommittee
Room W1312, Pocahontas Building
900 East Main Street
Richmond, Virginia 23219

Senator Howell, Co-Chair
Senator Hanger, Jr., Co-Chair
Senate Finance and Appropriations Committee
Health and Human Resources Subcommittee
14th Floor, Pocahontas Building
900 East Main Street
Richmond, Virginia 23219

Re: DMAS Fast Track Drugs and Emerging Therapies Work Group

I am writing today on behalf of the National Organization for Rare Disorders (NORD), regarding the Department of Medical Assistance Services (DMAS) Fast Track Drugs and Emerging Therapies Work Group (Work Group) and the Work Group’s final report expected in December.

Since its founding in 1983, NORD has been an independent advocacy organization dedicated to patients and families affected by rare diseases and the organizations that serve them. NORD, along with its more than 300 patient organization members, is committed to the identification, treatment and cure of the more than 7,000 rare diseases, of which approximately 90% are still without a U.S. Food and Drug Administration (FDA) approved treatment or therapy. Therefore, it is vital that when a therapy is approved by the FDA, it is made available to patients as quickly as possible.

Patient Participation in the Work Group

NORD recognizes the administrative complexity of developing appropriate coverage criteria for new drugs. However, we are concerned that this Work Group, which has been tasked with making “recommendations regarding the timeline for providing coverage from the date of FDA approval” lacks representation from any patient or patient organization.¹ The 2020 Virginia Acts of Assembly Chapter 1289, Item 313.CCCCC, which creates this Work Group, does not specify the inclusion of patients or caregivers, however it does direct DMAS to include any necessary stakeholders. NORD believes this group should include patients. The value of incorporating patient experiences and perspectives into these recommendations cannot be overstated. For many rare disease patients, including children, Medicaid coverage is a lifeline that provides access to necessary medications. The lack of a patient

¹ Meeting of the Fast Track Drugs & Emerging Technologies Workgroup. (August 25, 2020) Retrieved at:https://townhall.virginia.gov/L/GetFile.cfm?File=Meeting%5C64%5C31252%5CMinutes_DMAS_31252_v1.pdf

representative on this Work Group is a significant oversight and we are concerned that the group may release recommendations that, if implemented, would not be reflective of the needs of the rare disease community and could ultimately be detrimental to their health.

Fast Track & Emerging Technologies

NORD is especially concerned that this Work Group may recommend arbitrary and harmful delays in coverage for drugs that have been approved under one of the FDA's expedited review programs. There are many conditions in the rare disease community for which expedient access to approved treatments can prevent irreversible harm, and possibly even death. For these patients, the Commonwealth should be doing everything possible to reduce the amount of time between FDA approval and coverage in a responsible manner. For example, Michigan, which was one of three states examined by the Work Group during a meeting on August 25, imposes an automatic coverage delay of 6 months after FDA approval for all drugs. This delay can be devastating to rare disease patients.

In our review of Item 313.CCCCC, and in slides from the August 25, 2020 Work Group presentation, we are concerned about the Work Group's understanding of the various FDA expedited approval programs. Considering the critical importance of these programs to the rare disease community, we would like to take this opportunity to provide clarification regarding these programs.

Under Item 313.CCCCC, the charge of the Work Group is "...to assess policies and procedures, including risk sharing arrangements, reimbursement methods or other mechanisms to determine Medicaid coverage and reimbursement of FDA fast-track drugs and emerging break-through technologies." The August 25 slides refer to "FDA definitions" of the terms "Fast-Track" and "Emerging Technologies."² However, this slide excerpts only a small part of the FDA definitions and fails to provide a complete overview of the benefits awarded to products selected for these programs. FDA awards these designations to certain drugs and biologics only if they meet the criteria set forth in law and regulations, entitling them to benefits including more frequent communications and meetings with the FDA.

FDA permits products to use the "Fast Track" process if they are therapies that treat a serious medical condition, address an unmet need, and provide a significant improvement in safety or efficacy over existing therapies.³ The benefits of this process are more frequent communication (such as more in-person meetings and written correspondence between the Sponsor of a drug and the FDA) and rolling review, where the FDA may begin reviewing an application as the various steps are completed, rather than waiting until the entire application is complete.⁴

The "Breakthrough Therapy" designation, or "Emerging Technologies" according to the Work Group's documents, also requires that the drug or biologic (notably not a device) in question treat a serious condition and address an unmet medical need. It also requires that clinical evidence shows that the

² Slide 6 of August 25, 2020 slide presentation.

³ FDA. (January 4, 2018) Fast Track. Retrieved at: <https://www.fda.gov/patients/fast-track-breakthrough-therapy-accelerated-approval-priority-review/fast-track>

⁴ Ibid

therapy is a substantial improvement over existing therapies which, in most cases, is represented by an improvement of a patient's irreversible morbidity or mortality.⁵ The benefits of the Breakthrough Therapy designation are similar to the Fast Track in that both receive more frequent communication with FDA and rolling review. However, Breakthrough Therapy designation allows the Sponsor of the product to meet with FDA at an earlier stage of the review process and grants further Agency commitment to expeditiously reviewing the product.⁶ The approval granted by the FDA for these designations is indeed final, demonstrating that these therapies have met the gold standard of FDA approval for safety and efficacy. The assertion that there is another round of review by the FDA for the therapies that utilize these mechanisms is unfounded. NORD is particularly concerned by any proposals that create barriers to access for drugs that utilize an expedited approval program, as these therapies treat diseases that are, by definition, serious and life threatening.

One final distinction that NORD would like to point out is that Fast Track and Breakthrough Therapy are only two of several processes or designations that have been established to expedite the approval of drugs. Other programs include "Accelerated Approval," "Priority Review," and the "Regenerative Medicines Advanced Therapies Designation."⁷ Therefore, it is important the Work Group recognize and understand that their scope, as they have defined it, only applies to a very narrow group of drugs or biologics, not devices, that received the designation rather than all FDA reviewed products that utilize expedited approval mechanisms.

In short, the FDA's Fast Track and Breakthrough programs require an increased dedication of resources by FDA; these programs do not result in a different or lower approval standard.⁸ These processes still require the FDA to review and, if appropriate, approve the drug by applying the same standard of safety and effectiveness mandated under the Federal Food, Drug and Cosmetic Act as all other therapies that seek FDA approval.⁹ Congress enacted the laws directing FDA to establish these programs out of a recognition of the potential scientific advancements and positive outcomes for patients that come from additional efforts by the agency to expedite the review and provide greater regulatory guidance for sponsors of these products. They are not an indication that the threshold for approval for these drugs is somehow lesser than products approved through non-expedited pathways.

Conclusion

The core premise of FDA's expedited programs, as established by Congress, is that these drugs are exceptionally important to patients and should be provided to them in a manner that is as expedient as possible. Unnecessary delay in patients access to these products runs counter to the clear intent of the laws that establish these programs.

⁵ FDA. (January 4, 2018) Breakthrough Therapy. Retrieved at: <https://www.fda.gov/patients/fast-track-breakthrough-therapy-accelerated-approval-priority-review/breakthrough-therapy>

⁶ Ibid

⁷ FDA. (February 23, 2018) Fast Track, Breakthrough Therapy, Accelerated Approval, Priority Review. Retrieved at: <https://www.fda.gov/patients/learn-about-drug-and-device-approvals/fast-track-breakthrough-therapy-accelerated-approval-priority-review>

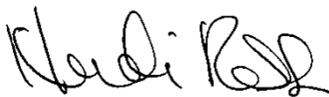
⁸ 21 U.S.C. § 356

⁹ 21 U.S.C. § 355

NORD thanks the Chairs for their consideration of this letter. The last decade has seen a tremendous growth in new, innovative therapies for rare disease patients and many rare disease patients who were once without any possibility of receiving targeted treatments for their condition may soon have new therapeutic options.¹⁰ As such, it is important to NORD that the Work Group carefully consider the patient impact of their recommendations. We reiterate our concern that the patient perspective has been absent from the deliberations of the Work Group thus far, and request that the final report be shared with NORD, and other members of the patient community, prior to publication. NORD urges the Work Group to reconvene with additional stakeholders present to represent the perspective of the patients who rely on access to these critical drugs.

We look forward to continuing to work with your committees to improve access to sustainable and affordable treatments for our patients. For questions regarding NORD or our comments please contact Corinne Alberts at calberts@rarediseases.org. Thank you for your consideration.

Sincerely,

A handwritten signature in black ink that reads "Heidi Ross". The signature is written in a cursive, flowing style.

Heidi Ross, MPH
Director of Policy

¹⁰ According to a recent study authored by Tufts Center for the Study of Drug Development, the share of new drug approvals worldwide for rare diseases doubled from 29% of all approvals in 2010 to 58% in 2018.