April 28, 2017

The Honorable Lamar Alexander, Chairman
U.S. Senate Committee on Health, Education, Labor and Pensions
428 Dirksen Senate Office Building
Washington, D.C. 20510

The Honorable Patty Murray, Ranking Member
U.S. Senate Committee on Health, Education, Labor and Pensions
428 Dirksen Senate Office Building
Washington, D.C. 20510

Re: NORD Comments on the FDA Reauthorization Act of 2017 Discussion Draft

Dear Chairman Alexander and Ranking Member Murray:

On behalf of the 30 million Americans with one of the nearly 7,000 known rare diseases, NORD thanks the Senate Committee on Health, Education, Labor and Pensions (HELP) for the opportunity to provide comments on the discussion draft of the *FDA Reauthorization Act of 2017* (S.934).

NORD is a unique federation of voluntary health organizations dedicated to helping people with rare "orphan" diseases and assisting the organizations that serve them. NORD is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and patient services.

I. The Importance of FDA User Fees:

First and foremost, the passage of the user fees included within the *FDA Reauthorization Act of 2017* are incredibly important to ensuring the FDA has the capability to hire and retain expert staff to review orphan therapies. It is critically important to rare disease patients that the FDA is well funded and adequately staffed in order for innovative orphan drugs and medical devices to reach our patients as quickly as possible.

To quote from a statement recently submitted to the Senate HELP Committee and House Energy and Commerce Committee (in concert with Friends of Cancer Research):

> "Our request of the Committees of Jurisdiction and Congress as a whole is simple: please keep the user fee reauthorization process non-partisan, uncontentious, and focused on the patients FDA serves every day.
>
> The FDA largely relies on user fees authorized by Congress to operate. Without the user fees, a majority of drug, biologic, and device reviewers would be laid off, and the necessary review of innovative therapies would be substantially impaired if not halted all together.
>
> The user fee acts are far too important to jeopardize with controversial partisan policy topics. We recognize the desire for additional reforms related to therapeutic development incentives, review,

and access. But we respectfully request that attempts to reform these areas without full bi-partisan support are not pursued as part of the UFA reauthorizations.

The cancer and rare disease patient communities rely on FDA to ensure that innovative, safe, and effective treatments reach those in need. We thank the HELP and E&C Committees for moving forward with these critical funding mechanisms, and look forward to their swift and unimpeded passage."

For these reasons, we support the user fees enumerated within the discussion draft of the *FDA Reauthorization Act*. In addition, while we understand the desire of many to pass a "clean" user fee reauthorization bill, we believe several policies negotiated as part of the PDUFA and MDUFA Committee Goals Letters should be included in the next draft of the *FDA Reauthorization Act of 2017*.

II. Proposed PDUFA VI Recommendations:

The PDUFA VI Commitment Goals Letter builds upon the successes of the PDUFA V agreement in many ways. Below are our comments on the specific sections of the Goals Letter that we hope Congress enacts within FDARA. These comments were originally submitted to FDA in response to the publication of the agreements, and are organized to reflect the structure of the letter.

D. Enhancing Regulatory Science and Expediting Drug Development:

2. Breakthrough Therapies:

NORD supports the addition of resources to implement the breakthrough therapy program. As an added policy attached to the PDUFA V agreement, the breakthrough therapy program did not receive the required resources to ensure the FDA could appropriately implement the program. With over 400 breakthrough designation requests, it is clear the breakthrough program needs additional dedicated PDUFA user fees.

The breakthrough therapy pathway is particularly important for innovative orphan therapies. With breakthrough's "all hands on deck" approach, therapies that receive a breakthrough designation gain critical FDA attention and assistance in moving through the development and review process. However, with 46 breakthrough therapies approved by the FDA as of July 18, 2016, only five were for rare inherited disorders.[1]

With the additional funds included in the Goals Letter, we hope the breakthrough therapy pathway can be opened to more orphan therapies for rare inherited disorders.

3. Early Consultation on New Surrogate Endpoints:

Biomarkers and surrogate endpoints represent some of the most promising innovative drug development tools. Rare diseases that had previously been thought of as unsolvable and untreatable could receive

[1] Friends of Cancer Research. 2016. "Breakthrough Therapies. July 18, 2016. Accessed August 22, 2016. http://www.focr.org/breakthrough-therapies?title=&field_sponsor_value=&field_therapy_category_tid%5B%5D=45&field_fda_status_value%5B%5D=1

renewed attention with the advancement of the use of biomarkers or surrogate endpoints in clinical development.

Still, with so little known about the scientific underpinnings and disease progression in rare diseases, there is much progress still needed to be made in order to allow rare disease development to truly benefit from the use of biomarkers and surrogate endpoints. By discussing the use of surrogate endpoints earlier and more often, this provision could assist orphan drug developers in the use of surrogate endpoints in their study design.

We support the enactment of this provision, and hope to work with the FDA in ensuring these early consultations are available to orphan drug developers.

4. Rare Disease Drug Development:

NORD thanks the FDA and industry for their commitment to ensuring thorough yet expedient review of orphan therapies. This commitment is evident in the proposed expansion of the Rare Diseases Program (RDP) staff into the CDER teams reviewing orphan therapies, and the continued training of CBER staff on "flexible and feasible approaches to review".

We are very supportive of this provision for several reasons. First, we still hear too often from our patient organization members about FDA inappropriately requiring particular trial designs. For example, our member organizations have expressed strong concerns about participating in extra confirmatory trials, being placed on a placebo control, or other forms of perceived inappropriate controls, and various other particularly arduous trial design requirements.

We also are aware of the wide spectrum of utilization of other flexible review techniques afforded by statute across review divisions for orphan drugs. Many review divisions are proficient in appropriately using expedited review pathways, accepting small trial sizes, using biomarkers or surrogate endpoints, or various other regulatory flexibility tools. Unfortunately, many other review divisions are not.

Finally, we continue to observe review divisions inappropriately deemphasizing quality-of-life improvements. Even if a therapy misses a primary endpoint, or perhaps a secondary endpoint, the quality-of-life improvements brought by that therapy still must be considered. Review divisions also have required particularly difficult endpoints to achieve, especially considering the small patient populations involved.

We believe each of these failures to appropriately use flexible regulatory review for orphan therapies arises from a fundamental misunderstanding of the rare disease patient experience. Rare disease patients are unique. They have a different risk/benefit formula than the general population. Their willingness to accept certain side effects is often misunderstood, and their prioritization of which symptoms to treat is unique. In addition, travel and participation in trials is often more difficult. We also believe that rare disease patients, particularly children, should not be required to go on a placebo and off of the therapy that is currently sustaining them.

All of these unique qualities need to be considered by review teams when reviewing orphan therapies. By placing RDP staff in each orphan drug review, these rare disease patient perspectives can be reflected and considered.

Any expansion of responsibility must be accompanied by a requisite expansion of resources and full-time employees (FTEs). The RDP already only has a handful of staff members, and to drastically expand their responsibilities as proposed in the Goals Letter without adding staff members would be irresponsible.

With added staff, we hope the FDA carefully considers how to assign RDP staff to particular reviews, and greater clarity on how FDA would do this is welcome. Given the broad spectrum of orphan therapies the FDA reviews each year, it may make sense to assign RDP staff to only one or a handful of review divisions rather than expecting them to participate in reviews across the disease spectrum. This would allow them to specialize in particular rare disease areas, and allow them to build an expertise in flexible review of therapies in certain disease areas.

This would also allow the RDP staff to proactively engage with the rare disease patient communities that may have therapies moving their way through the FDA review or pharmaceutical pipeline. While the collection of patient perspective data through the Patient-Focused Drug Development (PFDD) initiative and other avenues is ideal, the vast majority of rare disease patient organizations may be too small to undertake such a resource intensive endeavor.

We hope that RDP staff that specialize in certain disease areas can get to know the patients, their families and caregivers, and the organizations that represent them in order to have an understanding of the disease experience, the patient's risk/benefit formula, and various other important considerations only the patient can provide. This task may include RDP staff traveling to family conferences, both in and outside of Washington, D.C., as well as various other opportunities to get to know the patients.

With these experiences and connections in hand, we believe the additional RDP staff are better equipped to facilitate the flexible review of orphan products.

5. Advancing Development of Drug-Device and Biologic-Device Combination Products Regulated by CBER and CDER:

NORD supports the investment of resources in the combination product review process, and is particularly supportive of the investment to the Office of Combination Products as well as each Center that plays a role in combination product review. With PDUFA's investment, we hope this provision will facilitate the review of both drug-led and device-led products, and does not lead to a situation in which drug-led products (PDUFA-led products) are afforded greater attention and resources compared to their device-led counterparts.

6. Enhancing Use of Real World Evidence for Use in Regulatory Decisionmaking:

We support the enactment of this provision, and thank the FDA and industry for their commitment towards advancing innovative opportunities to assess a therapy's safety and effectiveness. With the inherent difficulty of generating statistically significant conclusions from orphan drug clinical trials, the

advancement of inclusion of real world evidence in regulatory decision making could greatly benefit the rare disease patient community.

8. Enhancing the Incorporation of the Patient's Voice in Drug Development and Decisionmaking:

One of NORD's primary regulatory priorities for many years has been the incorporation of the rare disease patient's perspective and experience into drug development and review. We were supportive of the creation of the PFDD initiative as part of PDUFA V, and have since supported the FDA in its efforts to expand PFDD opportunities to additional patient organizations through the creation of the externally-led PFDD meeting initiative and support for individual-disease guidance development.

We believe the proposals contained within the Goals Letter are a natural next step, and we are pleased to support their enactment. We hope these proposals will further encourage and facilitate orphan drug developers to include the patients and their perspective throughout the development of the therapy, and we hope to one day see each and every orphan drug that reaches the market reflect exactly what that specific patient population desires in a therapy.

To successfully reach this goal, we hope this provision will be implemented in the following ways. First, we strongly encourage the FDA to make any and all instructive guidances, public meetings, or other documentation to be accessible and digestible to the full spectrum of rare disease patient organizations. Only a handful of our 260 member patient organizations have the regulatory sophistication and dedicated resources to hold their own external PFDD meeting, conduct a scientific meeting with the FDA, or articulately participate in guidance development. We hope FDA considers the small individual rare disease patient organizations and their limited capacities when putting forth materials to ensure all patients and their organizations can equitably participate.

Second, we hope the FDA will continue to encourage and support patient organizations in their conducting of externally-led PFDD meetings and creation of guidance for industry for their particular disease. We understand that we should move on from the over 20 FDA-led PFDD meetings, but we still believe the externally-led meetings and guidance development are great opportunities for our members. This being said, as already discussed, we also hope FDA will put forward additional opportunities for smaller organizations to participate who do not have the resources to conduct PFDD meetings or develop guidance for industry.

Finally, we understand some in the patient community are concerned with the timeline put forth for guidance development within this section. We certainly sympathize with the FDA's resources (or lack thereof), both financially and staff expertise-wise, to implement these sections. We hope this PDUFA agreement will result in the substantial increase in both the number of FTEs working on patient input as well as the expertise they bring.

We hope that if the FDA implements this section using the timelines proposed, that the FDA will advise organizations such as NORD and others who are actively working on the science of patient input on how and where to make progress in the meantime.

9. Enhancing Benefit-Risk Assessment in Regulatory Decisionmaking:

NORD supports the continuation and expansion of FDA's "enhanced structured approach to benefit-risk assessment". Rare disease patients' benefit-risk formulas are the likeliest to be misunderstood or misconstrued due to their small, dispersed, and heterogeneous patient populations. By investing in FDA's structured benefit-risk assessment development, the FDA and industry are committing to the better and more representative inclusion of rare disease patients' benefit-risk assessment. We support the enactment of this provision.

10. Advancing Model-Informed Drug Development, and 11. Enhancing Capacity to Review Complex Innovative Designs:

We are supportive of both of these provisions as orphan therapies often require innovative and complex trial designs in order to show safety and effectiveness. For these reasons, the advancement of model-informed drug development could open the door for rare disease therapeutic development that may not have moved forward otherwise.

Industry's commitment to enhancing the FDA's capabilities in reviewing complex innovative designs is important in ensuring this occurs, and we are thankful to industry and FDA for prioritizing this burgeoning area.

13. Enhancing Drug Development Tools Qualification Pathway for Biomarkers:

The strengthening of FDA's biomarker qualification process is an important priority for NORD. As already discussed, we view the use of biomarkers and surrogate endpoints as impactful drug development tools that could greatly spur development in rare diseases that are difficult to develop in. The public meeting, guidance development, and website creation will all facilitate the process for biomarker qualification, and hopefully eventually lead to the use of biomarkers in orphan drug development.

G. Improving FDA Hiring and Retention of Review Staff

As a founding member of the Alliance for a Stronger FDA, no one is more supportive than NORD in ensuring FDA is adequately funded, and is able to hire and retain high quality candidates. Special expertise is needed to appropriately review orphan therapies, and it is important to NORD and rare disease patients that the FDA has the capabilities in house to appropriately review orphan therapies.

III. Proposed MDUFA IV Recommendations:

In addition to important provisions within the PDUFA VI Commitment Goals Letter, additional impactful reforms are included within the MDUFA IV Commitment Goals Letter. We hope both of these proposals are also included within the next iteration of FDARA.

L. Patient Engagement:

NORD is thankful for the inclusion of this proposal to facilitate greater Agency actions to "advance patient input and involvement in the regulatory process".

Greater patient involvement in the medical device development and review process is one of NORD's main priorities for the MDUFA reauthorization as discussed in our July 2015 public statement, and reiterated throughout the monthly stakeholder meetings. We believe that patients and patient organizations need to be fully integrated into the device development and review process in order for more patient-centric devices to reach rare disease patients.

We believe this provision will successfully move us forward in this direction. FDA will build its staff capacity and expertise to incorporate and review patient perspective information (PPI) and patient reported outcomes (PROs) through additional MDUFA-supplied resources. The FDA will also hold one or more public meetings to facilitate public discussion and feedback on the generation and integration of PPI and PROs.

In order to focus the FDA efforts within this initiative, FDA proposes to "identify priority areas where decisions are preference-sensitive and PPI data can inform regulatory decision-making, in order to advance design and conduct of patient preference studies in high impact areas". While we understand the need to focus efforts to the areas of greatest potential benefit, we request that FDA do so incredibly carefully. Too often rare diseases have been forgotten due to the small patient populations and limited advocacy resources.

If the FDA moves forward with identifying priority areas for PPI development, we request that the FDA put forward extensive and easily digestible information and instructions for disease areas or patient populations that may not have been identified as a priority. Potentially analogous is the Patient-Focused Drug Development (PFDD) Initiative administered by the Center for Drug Evaluation and Research (CDER) and the Center for Biologics Evaluation and Research (CBER). The PFDD Initiative will hold twenty-four public meetings on specific diseases or disease areas, and has also put forward instructions for patient organizations to move forward with their own meetings.

We hope FDA will similarly ensure that patient organizations can fully take advantage of the new FDA offerings and information on PPIs and PROs even if their disease area is not identified as a priority.

Similarly, we request that FDA ensure that all information put forward on how patients and patient organizations can participate within this initiative is accessible and digestible to the full spectrum of regulatory sophistication within the patient organization community. Approximately seventy percent of NORD's 260 member rare disease patient organizations have fewer than five full-time employees, and they likely have very limited resources to dedicate to regulatory engagement and expertise development or investment.

For this reason, we request that FDA put forward resources with patient organizations of all sizes and regulatory capabilities in mind in order to ensure that all rare disease patients and their organizations have an opportunity to benefit from this exciting initiative.

Finally, with similar initiatives on patient preference information proposed as part of the Prescription Drug User Fee Act (PDUFA) reauthorization, we hope these initiatives will develop consultatively and collaboratively to ensure they work in harmony, and patients and patient organizations are not presented with confusingly disparate opportunities.

M. Real World Evidence (RWE)

We thank FDA and the regulated industry for the inclusion of this provision on real world evidence. Rare diseases are nearly uniformly under studied and investigated resulting in a dearth of data on the treatment of rare diseases. Through the MDUFA investment in the National Evaluation System for health Technology (NEST), additional data on the use of devices in rare diseases can be collected.

This data could prove incredibly valuable. Many rare disease patients use a device off-label because that particular device was never studied in the rare disease population prior to marketing approval. Through the collection of real world data in these populations, indications could be expanded, and rare disease indications could be added using real world evidence.

Finally, we request patients and patient organizations to be formally included within the infrastructure of the NEST. We understand it is the intention for patients to be well represented on the board of the NEST, but we request formal assurance that patients and their organizations will be represented.

IV. Additional Areas to Consider:

As further iterations of FDARA are considered, NORD would like to propose additional policies for consideration:

FDA Office of Patient Affairs:

With the advent of new and innovative patient engagement programs within the Food and Drug Administration (FDA), there is a growing need for greater coordination of FDA patient interaction and input. Improved coordination can be achieved by creating an office that will centralize engagement and coordinate opportunities for patient involvement. The Office of Health and Constituent Affairs has a similar directive, but lacks the clear mandate and adequate resources to effectively facilitate patient involvement within FDA.

The FDA is already exploring this opportunity. On March 14th, 2017, FDA put forward a Request for Comment on the creation of an Office of Patient Affairs, "which will be tasked with supporting and coordinating patient engagement activities across medical product centers and other offices that engage with patients and their advocates on matters pertaining to medical products".

While we adamantly support the FDA's endeavor, additional legislation is needed to ensure the Office has the resources and authorities needed to successfully carry out their mission. This proposal and accompanying legislative text statutorily creates the Office of Patient Affairs within FDA Office of Medical Products and Tobacco. We believe the best and most efficient way to do this is to simply move the Office of Health and Constituent Affairs to this new role.

The responsibilities of this Office include:

1. **Assist Patients and Physicians Seeking Single-Patient Expanded Access Requests:** With better visibility, accessibility, and ability to work across the Centers, this Office will serve

as the main point-of-contact for incoming single-patient expanded access questions and requests. The Office will include dedicated staff, known as the Expanded Access Coordination Program, to provide education and information to patients and caregivers on the regulatory framework for single-patient expanded access requests, and also to serve as the liaison to the appropriate officials within the Agency for completing the request. This Office will also keep a database of expanded access requests and FDA's decision on the expanded access request.

This Office will not have any additional regulatory authority on expanded access requests, and the decision to grant a request will remain in the hands of the review divisions. Instead, this Office will simply streamline the process in order to make it more transparent for patients and their caregivers.

2. Ensuring Patient Participation Opportunities in the Drug, Biologic, and Medical Device Review Processes: With the creation of this Office, patients will be better able to take advantage of existing patient involvement opportunities. For example, this Office will more effectively recruit Patient Representatives to serve as Special Government Employees (SGEs).

This Office will provide a centralized navigation point for getting patient representative SGE applicants screened for COI. This Office will be charged with training and advising SGE candidates. Additionally, the Office will also be responsible for keeping a database of qualified patient representatives to take part in patient involvement opportunities.

Not only will this strengthened Office be valuable in carrying out FDA's current patient involvement opportunities, but it will also play a key role in implementing future patient involvement programs, including provisions of the 21st Century Cures Act, and various other FDA patient involvement proposals.

3. Internal Coordination across the Centers' Patient Engagement Initiatives: This Office will be tasked with fostering collaboration across Centers on their patient engagement initiatives. Without usurping the authority of each Center to plan and implement its own patient engagement initiatives, the Office will assist the Centers in coordinating and collaborating, as well as sharing best practices, on patient engagement initiatives.

This can be achieved through the formation of an internal working group with representatives from the centers and offices across FDA. In fact, this group has already informally been created under the PLP. We envision this group to have various duties, including the discussion and sharing of patient engagement and involvement best practices across centers, the development of Cross-Center patient involvement opportunities, and the development of patient engagement models to be recommended to the Commissioner and public at large. Such a group will also be charged with providing the Commissioner with recommendations for improving patient involvement in the drug, biologic, and medical device development and review processes.

4. Educate Patients on Involvement Opportunities: This Office will represent FDA in outside meetings, public appearances, conferences, and other externally facing opportunities for FDA to proactively educate patients on involvement opportunities.

This Office will also be tasked with the development and upkeep of resources for patients to better educate themselves on FDA's regulatory framework, Agency programs and initiatives, and patient involvement opportunities. These resources include, but are not limited to, a patient-centered website and newsletter, similar to the current Patient Network Newsletter.

5. **Facilitate streamlined Conflict-of-Interest Determinations for Patient Representatives:** The Office could assist review divisions with conflict-of-interest determinations by conducting the competitor product analysis and alleviating any additional time and resource burdens of conducting these reviews. The Office can also assist in ensuring patient representatives are given COI waivers if there is compelling government interest for their participation.

6. **Report to the Commissioner, Public and Congress on Patient Engagement throughout the Centers:** The Office of Patient Affairs will be responsible for reporting regularly to the Commissioner and to the public on all patient engagement and involvement initiatives occurring within the Office and Centers. This Office will be responsible for reporting to Congress every two years on FDA's patient engagement initiatives, and the progress made within these programs concerning patient involvement. This two-year report should not be overly burdensome on FDA to produce, and should inform Congress and the general public of opportunities for patients to become involved in FDA practices. Additionally, this office will make data available to the public about patient involvement at FDA.

Office of Orphan Products Development:

NORD has been made aware of an increase in the number of orphan designation requests being made to the Office of Orphan Products Development (OOPD). It is our understanding that because of this increase in designation requests, and without the subsequent increase in staff capabilities or resourcing, the OOPD has had to extend its internal review goal from a 90 day review to a 120 day review.[2]

We find this additional delay troubling, and we request that OOPD is given the required resources to complete orphan designation determinations within the 90-day window. We understand that much of the funding for OOPD is provided by the user fees provided to the Office of the Commissioner. We hope the disbursement and distribution of such funding to the OOPD is reflective of the growing responsibilities they have incurred.

Regulatory Review of Certain Device-led Humanitarian Use Device and Drug Combination Products:

Drugs are approved based upon their safety and effectiveness (FD&C §505). Humanitarian Use Devices (HUDs) are devices that treat fewer than 4,000 individuals per year. HUDs are given a Humanitarian Device Exemption (HDE) from showing efficacy, and are instead approved based upon safety and probable benefit (FD&C §520). This is because the small patient populations make showing efficacy incredibly difficult.

[2] Gayatri Rao, 2016. "The Rise in Orphan Drug Designations: Meeting the Growing Demand". FDA Voice. July 18, 2016. Accessed August 22, 2016. http://blogs.fda.gov/fdavoice/index.php/tag/fdas-office-of-orphan-products-development-oopd/

In combination products, all components of the product must be approved using the same standard. Under statute, drugs may only be approved based upon safety and effectiveness. In a combination product that combines a drug and a humanitarian use device, the combination product must be approved based upon safety and effectiveness because the drug cannot be approved on any other standard.

If a HUD is a component of a combination product, it is required to meet the drug component's required safety and effectiveness standard. Effectiveness is very difficult for HUDs to demonstrate due to the economic and logistical barriers of studying small patient populations.

It is therefore very difficult for sponsors to show effectiveness in a HUD. Alternatively, a sponsor of a drug/HUD combination product could get each of the components approved individually, but this is economically and regulatorily infeasible.

This results in combination products with a HUD as a component simply not being developed due to the often unachievable effectiveness standard.

We believe there may be a simple solution. For drug/HUD combination products in which the drug component has already been approved by CDER or CBER on safety and effectiveness, the drug/HUD combination product will be approved based upon safety and probable benefit.

This targeted approach will allow drug/HUD combination products to reach patients without compromising their safety. Only drug/HUD combination products in which the drug has previously been approved in other routes of administration will qualify. Thus, there should be no concern regarding exposing patients to potentially unsafe or ineffective treatments.

NORD intends on continuing our advocacy for this targeted change as part of the MDUFA reauthorization process.

V. Right-to-Try and Access to Unapproved Therapies:

We strongly oppose the Trickett Wendler Right to Try Act, and we request Congress to stop any further considerations of this bill. First, we do not believe that enacting this legislation will aid patients in the way that it purports. Proponents of this legislation claim that by removing FDA from the process of obtaining unapproved therapies, patients will be able to receive potentially life-saving medications more easily.

This is simply not the case. For patients with immediately life-threatening illnesses who cannot participate in clinical trials, the FDA approves 99.5 percent of all expanded access requests submitted by physicians and companies. When access is denied, it is almost always the company that refuses to offer expanded access of their unapproved therapies for often very valid reasons. Removing FDA from the process will not result in an abundance of potentially life-saving medications.

Instead, this legislation could do significant physical and emotional harm to rare disease patients. Under this proposal, companies would only have to successfully complete a phase one trial and still be in active clinical development in order to sell their product. This legislation does not stipulate the product must be

moving forward into a phase two trial. Even if it did, phase one trials generally only test basic safety and toxicity in patients that do not have the disease the therapy may treat later on.

The notion that drugs that have completed a phase one study are safe is incredibly dangerous, and could lead to increased suffering or death. There is nothing in this legislation to prevent companies from developing products with no potential long-term prospects, solely with the objective of profiting off of patients desperate for a treatment. Instead, the bill opens the door to snake oil salesmen to once again take advantage of the desperate and suffering.

We believe that patients deserve to make their own choices, yet we also believe that patients deserve respect and dignity. We believe it is wrong for Congress allow swindlers to profit off of false hope. Instead, we believe Congress must protect patients from those who would seek to take advantage of them.

Second, we must balance the immediate need for treatment with the continued need for clinical research. Even if Right-to-Try succeeded in increasing access to unapproved therapies, it could still be damaging to further innovation. There are already so few patients in the rare disease community available to participate in clinical trials, and unfettered access to unapproved therapies outside of a clinical trial setting could be detrimental to full approval of innovative orphan drugs.

Finally, Right-to-Try creates a situation in which only wealthy patients could purchase investigational therapies and the poor are left out in the cold. This two-tiered system is completely unacceptable, and Congress must support policies that offer the same access to unapproved therapies regardless of income.

Right-to-Try is not the answer. Not only will it not increase access to innovative unapproved therapies, it will likely actually decrease such access as risk-averse companies will no longer be willing to offer their investigational therapy without the FDA as a partner. This legislation will only succeed in increasing access to unsafe, dangerous, and ineffective fabrications that could injure or even kill our patients.

Right-to-Try has no place within the user fee reauthorizations, and we implore Congress to stop all attempts to enact this harmful proposal.

We again thank the Senate HELP Committee for the opportunity to comment, and we look forward to working with Congress to ensure these vital user fee agreements and accompanying policy improvements are swiftly enacted. For questions on the above comments, please contact me at pmelmeyer@rarediseases.org.

Sincerely,

Paul Melmeyer
Director of Federal Policy