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Testimony

Senate Special Committee on Aging

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Chairman Collins, Ranking Member Casey and distinguished members of the Committee on Aging, thank you for allowing me to speak before you today. My name is Dr. William F. Harvey and I am a practicing rheumatologist at Tufts Medical Center in Boston, MA. In addition to my daily duties caring for patients with rheumatic and musculoskeletal disease, I participate in research into treatments for these diseases and work to develop information technology to better care for patients. I am also privileged to hold a volunteer position on the Board of Directors of the American College of Rheumatology (ACR), which represents approximately 9,500 rheumatologists and rheumatology health professionals. The ACR advocates for, among other things, affordable access to treatments for chronic conditions including Rheumatoid Arthritis (RA), Psoriatic Arthritis, Lupus, and many more. I wear on my lapel a bent fork, created by the ACR as a symbol to remind everyone that when you have arthritis, even simple tasks, like using a fork, can be difficult.

Recent advances in the treatment of Rheumatoid Arthritis and other diseases have created a "new normal" for patients suffering from rheumatic diseases. With early diagnosis and treatment the disability and disfigurement also symbolized by the bent tines may be prevented. A great tragedy emerging in our country is the increasing barriers to accessing these treatments, primarily related to their high cost. Before I discuss some of those barriers, I would like to share some background information.

Rheumatoid Arthritis is one of more than 100 auto-immune diseases. Auto-immune diseases are those in which the immune system attacks various parts of the body, instead of bacteria and viruses.

In the case of rheumatoid arthritis, the immune system attacks the lining of the joints, most often in the hands and feet. Rheumatoid arthritis is the most common auto-immune disease targeting the joints, affecting over 1.3 million Americans. It afflicts women2-3 times more frequently than men and has a peak incidence in middle age. However, it can be diagnosed at any age, and has a juvenile form. Because rheumatoid arthritis is a lifelong illness, there are many seniors today living with RA. Untreated, rheumatoid arthritis leads to significant joint damage, disability, and pain. Fortunately, today we have highly effective treatments for the condition.

Several decades ago, the mainstays of treatment were effective but highly toxic medications such as gold salts and steroids such as prednisone. While the latter is still used today, these medications do little to alter to progression of the disease and prevent disability. After advances in the 1980s, these medicines can effectively treat up to half of patients with RA. Examples of these disease-modifying anti-rheumatic drugs, or DMARDS, are hydroxychloroquine, leflunomide and methotrexate. These medications allow patients to get off the toxic steroids and prevent disability. Taken by mouth, they all currently have generic and brand name formulations and cost anywhere from a few dollars a month to several hundred. After application of insurance coverage, most patients pay co-pays less than 50 dollars each month for these medications. However, some patients will need additional medication to treat their disease.

After building on discoveries made initially by researchers funded by the NIH, pharmaceutical companies developed a new class of drugs called biologic DMARDs. They have the name “biologics” because they are proteins made from living organisms, and they are all administered via injection under the skin or by intravenous infusion. The ones available to inject under the skin may often be self-administered by the patient at home. Though designed in a laboratory, even today they are produced through a highly complex proprietary process involving using bacteria or other living organisms to produce copies of proteins that block parts of the immune system. Targets of these therapies include

tumor necrosis factor alpha (TNF-α), Interleukin 6, CD88, and CD-19, among others. These therapies are marketed by pharmaceutical companies under brand names, and there are currently 10 therapies in this class. Because of the complexity of production and the necessary quality control, these therapies are very expensive to study and produce. Consequently, their marketed are over $50,000 per year. Though highly effective, out-of-pocket costs to patients for these therapies can reach several hundred or even thousands of dollars per month. There are no generic versions of these treatments. Recently, biosimilars – which are not generics in the traditional sense because they are not exact copies of the original therapy – have begun to provide some alternatives.

Choosing the right therapy for a patient is a complex medical decision that considers other conditions and medications the patient may have, balancing the risk of side effects and many patient specific factors. Most physicians believe in the importance of shared decision making, which tailors treatment to the individual goals and concerns of the patient. Most rheumatologists start treatment with oral DMARDs and increase the dosage until the patient achieves remission or low disease state, which minimizes the risk of disability and pain. Using the lowest effective dose also limits potential toxicity of treatment, namely liver and other organ damage and infections. It may take 1-3 months, sometimes longer to find the effective dose for each medication tried. If the medication doesn't work, the patient will continue to have pain and disability, or require the use of toxic steroids until the right medication is found. If the oral DMARDs are ineffective, rheumatologists move on to the biologics. Factors to consider when choosing the right biologic therapy include medications already tried, history of infections and malignancy, and the ability to administer the medication to themselves. But overwhelmingly, the primary factor in the decision is which one is covered best by insurance. Because of their high cost every one of these therapies requires prior authorization before use, a process which can take days to weeks to complete before the patient can start treatment. The same 1-3-month trial and error may be necessary, as we do not have scientific evidence to determine which treatment will

work best for which patient; the result being that in may take months to get the disease under control. I will now provide some more detail about the difficulties in obtaining these treatments for patients.

I mentioned that each of these medications require a prior authorization. Each insurance company has a different set of forms requiring somewhat different types of information. Most require that the patient have their medication provided by a specific pharmacy. This is because the insurance companies negotiate, often through pharmacy benefit managers (PBMs), price discounts in exchange for preferred status on the insurance formulary. If a provider wishes to prescribe a medication that is not preferred, the prior authorization will be denied, and the provider must go through an escalating appeals process that may involve talking to a clerk, a pharmacist, a nurse, a "peer review" (another MD but typically not a rheumatologist), a same specialty review and, in the case of Medicare, an Administrative Law Judge. Each of these steps may take 10-30 minutes, during which time the provider is taken away from caring for other patients. Many practices have begun to employ at significant expense, other providers such as nurse practitioners, physician assistants and pharmacists to navigate this process. Without my pharmacist Jinkyu Lee, our resident "insurance wizard', we would drown in the administrative burden. To address this issue, I strongly endorse regulation requiring that insurance companies follow a standard, transparent process for documenting, evaluating and approving prior authorization requests. Every minute spent away from patients is a waste of providers' time and limits access for other patients. Policies requiring that a provider try therapies in a specific order are referred to as step therapy or fail first protocols. Since these are based primarily on cost rather than efficacy and shared decision making, I strongly endorse regulation requiring transparency of these policies and around the process by which providers may appeal to override them.

I mentioned the trend of using pharmacy benefit managers to address rising costs of therapies. At face value, this concept makes intuitive sense, allowing companies to negotiate the best possible prices for treatments. Some have advocated that Medicare exercise this same leverage. However,

while well-intended the use of PBMs in this way has led to an opaque process that favors maximizing payers' profits over shared decision-making utilizing that sacred bond between doctor and patient. Pharmacy benefit managers are for-profit companies that make their margin based on the difference between the list price and negotiated price. They benefit when the list price of a treatment is increased, as it improves their ability to drive margin. Because of contract law, only the pharmacy benefit manager knows what the true difference between list price and negotiated price is. Perhaps most egregiously, pharmacy benefits managers and insurers are not required to pass negotiated savings on to their beneficiaries in the form of co-pay discounts, and in my experience in most cases they do not. I strongly endorse requirements for increased transparency for pharmacy benefit managers – starting with properly defining terms like rebates – and for passing savings directly on to consumers.

Out-of-pocket expenses for patients are, as I have noted, substantial for these essential therapies. In addition to the issues noted above I wanted to discuss co-pays specifically. They were originally conceived to require consumers, patients, to have some skin in the game. More expensive medications carry larger co-pays, thereby incentivizing patients to request cheaper alternatives. This works well when considering typical, low-cost medications such as cholesterol, blood pressure and even oral DMARD medications. However, the system breaks down when applied to biologics for rheumatoid arthritis and other diseases. There are no generic alternatives and virtually all patients taking this medication have already tried and failed the cheaper oral therapies. Patients did not choose their disease, nor do they control the high cost of developing and marketing the only therapies left to treat their condition. Asking them to pay their 'fair share' is immoral and indefensible when it leads to medical bankruptcy. Further, these patients are more likely to leave the workforce, ending up on government subsidized disability, which further burdens the federal government. I can say unequivocally that many patients with rheumatoid arthritis or its close cousin arthritis associated with psoriasis, that many patients have no skin left to give.

For patients on commercial insurance, most pharmaceutical companies offer co-pay assistance and other support on an income adjusted basis. Medicare patients are prohibited by law from accessing this support. There are some private foundations that can support Medicare beneficiaries, but they are underfunded and not universally accessible. For these beneficiaries the only affordable option is to avoid self-injectable treatments, which fall under Part D pharmacy benefits and are therefore subject to copayments and the Medicare doughnut-hole phenomenon. The option in that case are those therapies which can be administered in a doctor's office or hospital by intravenous infusion and fall under the Part B benefit with lower out-of-pocket costs. This adds administrative and hospital costs to the drug costs, raising the total cost of treatment particularly in the hospital setting. But to me, and other rheumatologists, a patient who cannot afford to take their medication is an untreated patient, which we know will result in increased pain and disability. This puts providers in a very challenging position attempting to follow Medicare guidelines for appropriate use of Part B medication while making sure our patients have access to treatment. I can give many sad examples of patients under-dosing their medication to try to make their treatment more affordable, the result being suboptimal disease control and disability. For example, I have one patient who spread her injections out taking them every 3-4 weeks instead of every 2. We were both frustrated with her lack of improvement and it was only when I recommended trying another medication that she tearfully let me know what she had been doing. In another case, a patient called my office upset because she had spilled her medication. I was puzzled because it came in a pre-filled syringe. It turns out she was trying to inject half the medication to make it last longer. I strongly endorse legislation such as the Patient Access to Treatment Act (H.R. 2999) or other efforts that limit or cap out of pocket expenses for patients, so that these sorts of stories never happen again.

Lastly, I wish to briefly comment on the importance of biosimilars. These are biologic treatments which are similar to, but not exact copies of, existing biologics. A safe and vibrant biosimilar

marketplace is essential to the future of rheumatologic care. In Europe, where biosimilars have been introduced into the market earlier than in the U.S., they have seen an approximate 30 percent reduction in the total cost of the treatment. However, this level of reduction in cost did not occur until there were 3-4 competitor products in the market. The FDA has a process for approving biosimilars and two biosimilars to Remicade, a biologic DMARD are on the market. I encourage the committee to support adequate funding for the FDA to be able to rapidly but safely approve additional biosimilars. Unfortunately, other biosimilars approved by the FDA have been held up in patent litigation. Another panel member will address this issue, but I wish to reiterate generally that a vibrant biosimilar marketplace will lead to competition and reduced cost. As that happens, we must become vigilant about the drug distribution system and ensure that these savings are passed on to consumers, my patients.

I have covered many important issues with you and look forward to answering the committee's questions. I wish to thank the Chairman and Ranking Member for the opportunity to speak with you today. I have great faith in the institution of government and that its members will do everything in their power to protect the people of our nation who suffer from chronic disease such as rheumatoid arthritis and are burdened with the growing expense of treatment. These are not easy problems to solve. But the fact that we are gathered together today to focus on this issue is a testament to the people of our country that it is a set of problems worthy of solving. Together we can continue the conversation and search for solutions. Compared to two decades ago, I can look at a person newly-diagnosed with Rheumatoid Arthritis and tell them that their hands will never look like my fork. So long as they can access the revolutionary therapies that we know can prevent this progression. Together, we can un-bend the tines of my fork for current and future generations, so that they may remain healthy productive members of our workforce, and more importantly our families. Mothers and fathers can pick up their kids without pain and go to work without taking too many days off. Our seniors, who have

raised our nation and contributed so much, can be assured that they will be cared for without bankrupting themselves and their families. Scientific innovation has afforded our great nation so many opportunities, so long as they can access them. I look forward to working to solve those problems with you. Thank you again for accepting this testimony and I am happy to address any questions the Committee may have.

Respectfully submitted,

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