ALLIANCE for Regenerative Medicine

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PDUFA Reauthorization Public Meeting – July 15, 2015

Good afternoon. My name is Michael Werner. I am co-founder and executive director of the Alliance for Regenerative Medicine (ARM). It is my pleasure to be here today to participate in this public meeting. I'd like to thank the FDA for holding this meeting and giving me the opportunity to testify on behalf of ARM.

ARM seeks a regulatory pathway that is predictable and efficient to ensure safe and effective products reach patients as soon as possible. We know that FDA shares this goal. We are proud of our collaborative relationship with the agency and appreciate its support for regenerative medicine and other advanced therapies.

About ARM

Based in Washington, D.C., ARM is the preeminent global advocacy organization for the regenerative and advanced therapies community. ARM fosters research, development, investment and commercialization of transformational treatments and cures for patients worldwide. ARM empowers multiple stakeholders to promote legislative, regulatory and public understanding of, and support for, this expanding field.

ARM has more than 220 members worldwide. Prior to ARM's formation in 2009, there was no advocacy organization operating in D.C. that specifically represented the interests of the many stakeholders of this sector – the life sciences companies, research and academic institutions, patients and patient advocacy groups, investors and more.

My comments today can be summarized as follows:

1. Overall, PDUFA V has been a success. In particular, the expedited review provisions established during the formulation of PDUFA V have helped to speed to market important therapies.

2. Regenerative medicine and other advanced therapies hold the promise of treating many currently unmet medical needs and provide a major paradigm shift in medicine – treating the root causes of disease to stop or even reverse their progression. Many other countries have developed specific policies to support this sector. We call on FDA to take steps to ensure that these products reach patients as soon as possible.

3. ARM's specific ideas include:
   a. Establishment of a Standards Coordinating Body for these technologies.
   b. Creation of a Qualified Regenerative Medicine Product designation whose sponsor can receive assistance from FDA.

c. Improved coordination and communication among FDA review centers.

The Promise of Regenerative Medicine & Advanced Therapies

At present, the majority of treatments for many chronic and/or life threatening diseases are palliative – meaning that the method of care treats the symptoms without actually addressing the underlying cause, condition or disease. Our nation's healthcare system is correspondingly weighed down by costly, long-term treatments (compounded by a rapidly aging population), with few to no solutions for containing these rising costs.

The regenerative medicine and advanced therapies sector is truly the next frontier for patients, developing revolutionary treatments and potentially curing some of humankind's most devastating diseases – some of which are currently untreatable via conventional treatments – through the use of transformative scientific discoveries and technologies. These products are intended to augment, repair, replace or regenerate organs and tissues in the body and have already successfully treated certain cancers, chronic wounds, cartilage defects, complications from diabetes and other conditions.

Regenerative medicine and advanced therapies offer a significant improvement in the economics of current healthcare system – developing more effective treatments for the most burdensome diseases and conditions, helping patients lead longer, healthier and more productive lives while reducing the need for long-term and costly treatments.

Current focus areas include:

- CAR-T and other adoptive T-cell therapies to treat cancer.
- Using various gene therapy approaches to repair DNA in many genetic disorders and rare diseases, including sickle cell disease; hemophilia; beta-thalassemia; several forms of cancer, including leukemia; ophthalmologic diseases including dry and wet age-related macular degeneration (AMD); cardiovascular disorders; ischemic disease; neurodegenerative diseases such as Parkinson's, Huntington's and Alzheimer's; and more.
- Various cell therapy approaches in the treatment of many disease areas, including orthopedic, pulmonary, cardiovascular and dermatological conditions.
- Tissue engineering approaches for chronic wound care, organ regrowth and repair and tissue and cell scaffolding.

With more than 580 companies comprising the regenerative medicine and advanced therapies sector worldwide, this sector has moved from being characterized as an "emerging field" to one with clinical and commercial success and long-term viability. Globally, there are more than 60 approved and/or marketed products and more than 480 active clinical trials currently in Phase I, II or III.

The field is attracting a significant amount of investor interest, as well as a healthy partnering and collaboration environment, demonstrating the promise of the field. **In 2014, the sector raised $6.3 billion, representing a 112% increase over 2013.**

The Need For a U.S. National Strategy for Regenerative Medicine and Advanced Therapies

Although this sector is advancing, more needs to be done for it to reach its potential. Several other countries, including Japan, China, South Korea, the United Kingdom, and Canada, all have their own national regenerative medicine strategies, expediting products to patients and threatening to eclipse the U.S.'s leadership in this vital field.

For example, Japan has instituted a special regulatory approval pathway for regenerative medicine drugs that relies on early clinical trials data to ensure products reach the market quickly. The United Kingdom has developed special regulatory rules for these products under its Early Access to Medicine Scheme and the European Medicines Agency specifically formed the Committee for Advanced Therapies, focused on advanced therapy medical products (ATMPs) across the European Union.

ARM calls for a strong, federally-directed national strategy for regenerative medicine and advanced therapies to continue to move this sector forward and stay competitive on the global stage. Such a strategy includes federal agency coordination, support for research, capitalization for companies and regulatory reform. It also requires that FDA be staffed and resourced so it has the expertise to review the new products that are in the pipeline.

Previous PDUFA provisions, including the new expedited review programs, have been particularly useful at speeding access to new therapies. ARM is eager to build upon those and proposes the following:

- **U.S. Standards Coordinating Body for Regenerative Medicine & Advanced Therapies**
    - FDA has identified the lack of standards as an obstacle to drug development in this field. In response, ARM is working to develop a standards coordinating body for standards in regenerative medicine and advanced therapies. Various stakeholders, including NIST, FDA, private standard setting bodies, contract manufacturers, academia and others, have been engaged in this project.
    - This organization would work closely with global standards agencies, creating a central clearinghouse for the coordination, development, communication and implementation of technical and process standards and best practices for the regenerative medicine and advanced therapies sector.
    - Given FDA's longstanding work in – and support for – standards, we look forward to continued work with the agency on this project.

- **Improvements to approval pathways by facilitating the use of FDA incentive programs for Qualified Regenerative Medicine Products (QRMPs)**
    - To spur development of these important products in the U.S., we recommend that the FDA designate certain regenerative medicine and advanced therapy products as "Qualified Regenerative Medicine Products" or QRMPs. These are products intended for serious or life-threatening diseases with no currently available treatment options. QRMPs have the potential to modify the course of a disease or condition based upon clinical evidence that shows it may demonstrate substantial improvement over existing therapies. To facilitate review, FDA would meet with the sponsors of the QRMP to discuss expedited review opportunities.

- **Improved communications and coordination among FDA review centers**
    - Product developers find that coordination and communication between FDA review centers can be lacking. The result is delays in the process and increased development costs. This is a particular problem when FDA reviews combination products, though it occurs in other situations when more than one review center consults on an application.
    - ARM recommends the development of a review framework including timelines, communications and regulatory requirements that will facilitate consistency and efficiency among review centers.

Regenerative medicine and advanced therapies have already demonstrated their power to improve patient lives. With the potential to treat and even cure chronic conditions and diseases, these technologies hold enormous promise. Our goal is to work with FDA to make needed policy changes to support the development of these potentially life-altering and life-saving treatments.

Thank you for the opportunity to provide these comments. We look forward to working with you in the days ahead.