CBER Priorities

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CBER’s Proposed Strategic Goals

• **Goal 1:** Facilitate the development and availability of safe and effective medical products through the integration of advances in science and technology

• **Goal 2:** Conduct research to address challenges in the development and regulatory evaluation of medical products

• **Goal 3:** Increase preparedness for emerging threats and promote global public health

• **Goal 4:** Manage for strategic excellence and organizational accountability
Increased Activity in Gene Therapy

Investigational Drug Applications

Number of gene therapy IND applications to FDA is increasing noticeably and there are over 900 active INDs as of Dec 31, 2019

www.fda.gov

Emerging Infectious Diseases

Global Examples of Emerging and Re-Emerging Infectious Diseases

Source: National Institute for Allergy and Infectious Diseases

www.fda.gov
Center Priorities for 2020

- Develop a regulatory program for individualized (bespoke) therapies
- Cell therapy compliance (enforcement discretion ends Nov 2020)
- Address gene therapy human resource and infrastructure needs
- Advance pathogen reduction research
- Revisit blood donor deferral criteria
- Advance influenza vaccine manufacturing
- Foster global regulatory convergence for cell and gene therapies

Current Commercial Gene Therapy Manufacturing Capacity

Manufacturing capability is currently limiting the development and delivery of gene therapy

Approximate Treatment Population Per Year

- Viability? (Cost) 1-100
- Sweet Spot >100-10,000
- Viability? (Technology) >10,000
Vision for Facilitating Development of Individualized AAV Gene Therapies

- Develop a consortium (public-private partnership) to provide end-to-end solutions for key issues limiting the development and application of gene therapy to ultra-rare genetic abnormalities
- Anticipate that addressing need for individualized gene therapy products will lead to technical developments advancing the entire field