



Overview of Biological Products

FDA Basics Webinar
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Mantej (Nimi) Chhina, M.S., Ph.D.
Health Science Policy Analyst
Office of Medical Policy
Center for Drug Evaluation and Research
U.S. Food and Drug Administration

Objectives

- Biological products definition
- What is a “biological product”?
- Sources for biological products
- Types of biological products
- How are biological products different?
- Questions from consumers and patients

Definition of Biological Product

Section 351, Public Health Service (PHS) Act

- *(1) The term “biological product” means a virus, therapeutic serum, toxin, antitoxin, vaccine, blood, blood component or derivative, allergenic product, protein (except any chemically synthesized polypeptide), or analogous product, or arsphenamine or derivative of arsphenamine (or any other trivalent organic arsenic compound), applicable to the prevention, treatment, or cure of a disease or condition of human beings.*

42 U.S.C. § 262(i)

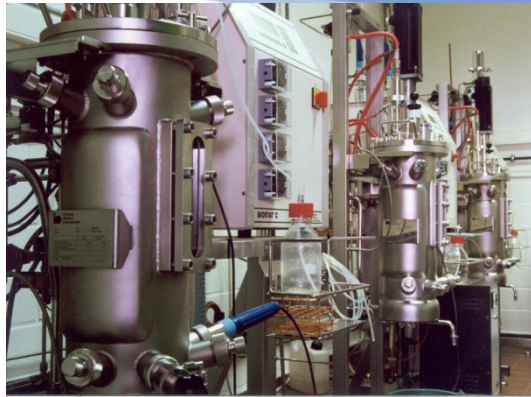
Examples of Biological Products

- Biological products are used for a wide range of diseases and conditions, including serious and life-threatening conditions such as cancers and rheumatoid arthritis. Some examples of biological products and examples of their approved uses include:
 - Botox: has both dermatologic and neurologic uses
 - Herceptin: for a certain type of breast cancer
 - Vaccines, for example: the Shingles vaccine and the flu vaccines
 - Enbrel: for rheumatoid arthritis and psoriasis

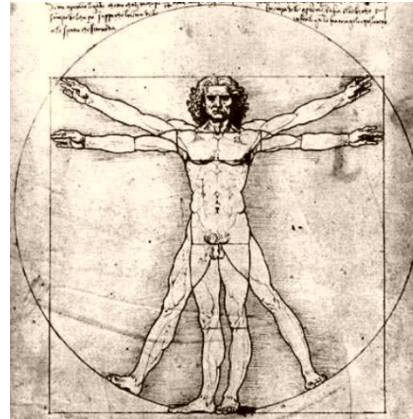
What is a Biological Product?

- Biological products, or biologics, are medical products.
- Biological products could be made of sugars, proteins, or nucleic acids or complex combinations of these substances, or may be living entities such as cells and tissues.
- Like drugs, biological products are used to either:
 - treat or cure diseases and medical conditions,
 - prevent diseases, or
 - diagnose diseases
- Biological products are made from a variety of natural sources.

Source Materials



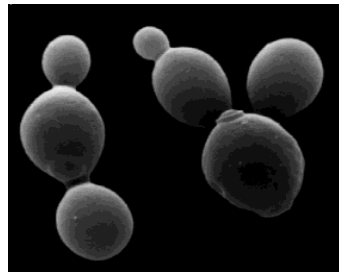
Mammalian cell-culture



Humans



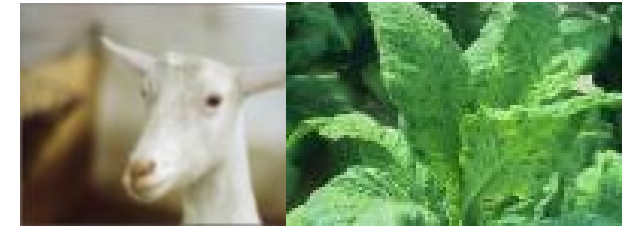
Avian cell-culture



Bacteria



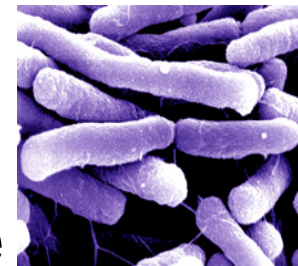
Mice



Transgenics



Plant cell-culture



Yeast



Insect cell-culture

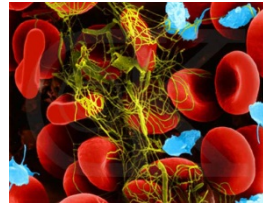
Types of Biological Products



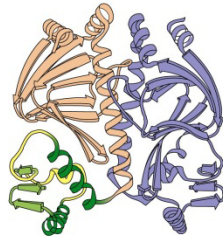
Blood Derivatives

Whole Blood

Blood Components



Proteins



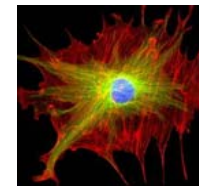
Human Tissues



Vaccines (preventive and therapeutic)



Allergenic Extracts



Cellular & Gene Therapies



Xenotransplantation Products

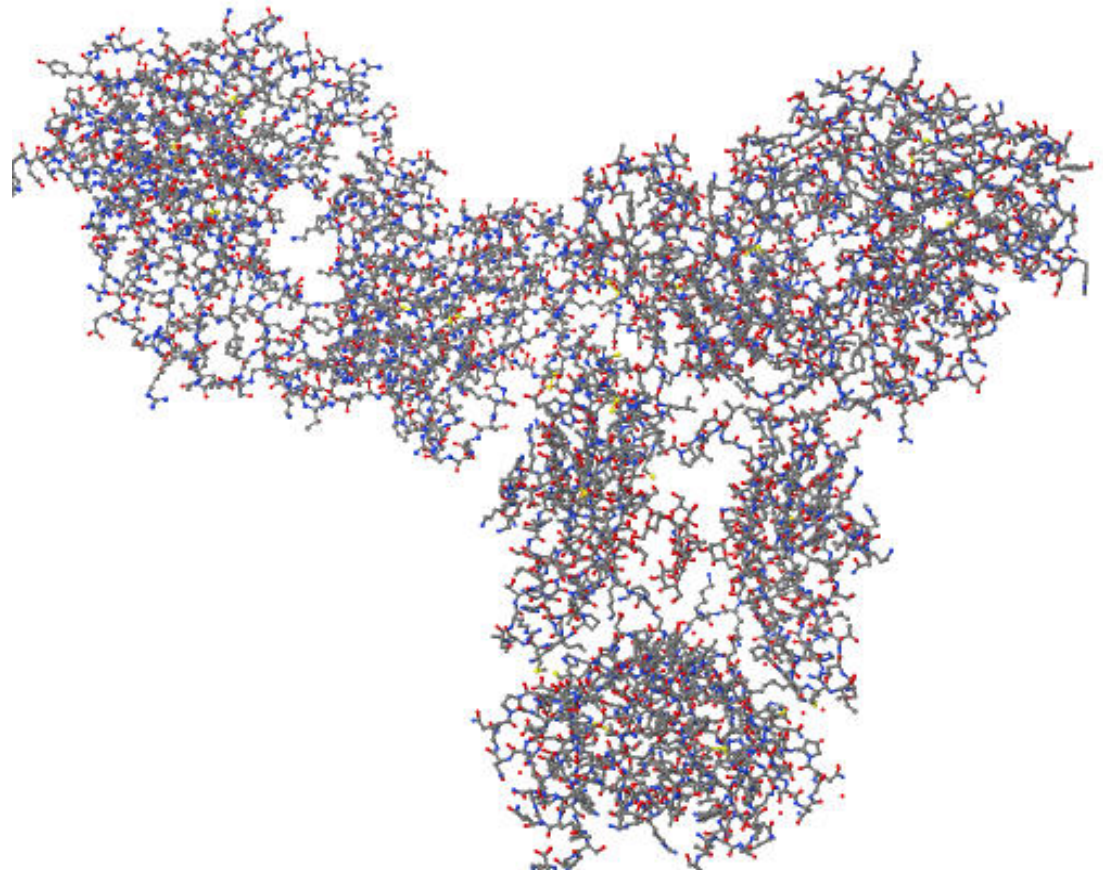
How are Biological Products Different?

Small Molecule Drugs	Biological Products
Generally low molecular weight	Generally high molecular weight
Usually organic or chemical synthesis	Made with/from live cells/organisms → <i>inherent & contamination risk</i>
Fewer critical process steps	Many critical process steps
Well-characterized	Less easily characterized
Known structure	Structure may or may not be completely defined or known
Homogeneous drug substance	Heterogeneous mixtures → <i>May include variants</i>
Usually not immunogenic	Often Immunogenic

Size and Complexity of Proteins

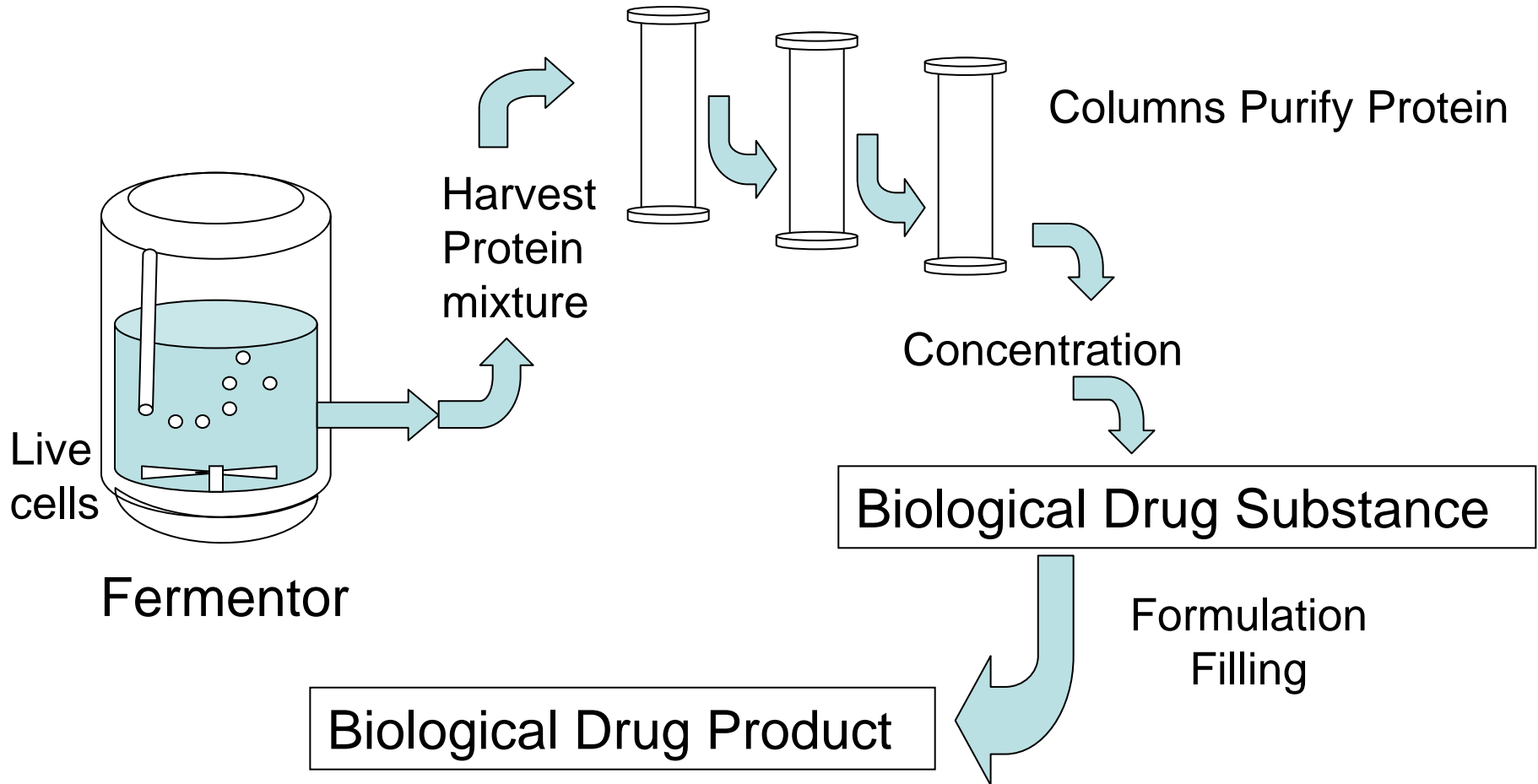


Aspirin 180 Da



Monoclonal Antibody ~150,000 Da

Example of a Biotechnology Process



Examples of Challenges for Biological Products

- Must be processed under tightly controlled conditions/controls throughout production to
 - consistently produce a safe, pure, and potent product, and
 - preclude the introduction of environmental contamination
- Biological products may be susceptible to extreme temperatures and light
- typically need refrigeration but may need frozen storage or preservatives
- shelf life may be limited

Bringing a biological product to the US market

- A manufacturer who seeks to market a biological product must submit a biologics license application (BLA), which includes:
 - Manufacturing information to demonstrate the company can properly and consistently manufacture the biological product
 - Data and information regarding the biological product (e.g., studies in animals and humans)
 - The company's proposed labeling for the biological product. The labeling provides necessary information about the biological product, including uses for which it has been shown to be effective, possible risks, and how to use it.

- The biological product is licensed and can be marketed in the United States only if
 - FDA determines that the data shows the biological product is safe and effective for its proposed use, and
 - the biological product can be manufactured in a way that ensures a quality product.

Summary

- Biological Products: diverse, complex products for the treatment, diagnosis and prevention of a broad range of common and rare diseases
- Complex processes for manufacturing, product testing, *and* for evaluation of safety and efficacy
- In contrast to most drugs that are chemically synthesized and have a known structure, most biological products are complex mixtures that are not as easily identified or characterized.
- Healthcare professionals and consumers can be assured that FDA will require licensed biological products to meet the Agency's exacting standards of safety and effectiveness.



Thank you!

Questions?



Remember to tune in for webinar on:

Biosimilar Biological Products

Monday, August 19, 2013

1:00 PM U.S. Eastern Time Zone