DPS

Presentation for:

PDA (Europe) Gene Therapy Roundtable

Berlin, Germany

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DPS Experience In Cell And Gene Therapy

- **Adeno Associated Viral Vector - DMD And Others (Clinical)**
- **Adenoviral Vectors – NSC Lung Cancer (Pivotal)**
- **Retroviral Vectors – Head And Neck Tumors (Clinical)**
- **Lentiviral Vectors – Multi-product/Suite Facility (Clinical)**
- **Autologous Transfected Epithelial Cell-based Vectors – Multiple Genetic Deficiencies (Late Clinical)**
- **Autologous And Allogeneic Stem Cell Therapy – Tissue Regeneration**

Design Considerations For Clinical And Pivotal Cell And Gene Therapy Facilities

- **Parallel Process Line Segregation And Changeover (Modular?)**
- **Containment And Safety (BSL-2 and 3) (Modular?)**
- **Aseptic Operation/Closed Systems**
- **Quality Control/Testing**
- **Single Use Equipment And Systems**
- **Robotics/Automation (Discrete Containers)**
- **Plasmid Production (Make Or Buy?)**
- **Exotic Unit Operations (Scalability?)**
- **Sterile Filling And Finishing (In-house Or Contract)**
- **Process Yields (Cell Culture Titers/Purification Yields)**
- **Control And Data Acquisition (Batch Records)**

Core Production Suite Productivity

- **Gene Therapy (rAAV) – 200 Sq. M. – 12 patients/yr**
- **Gene Therapy (rAAV) – 350 Sq.M. – 12 to 20 patients/yr**
- **Gene Therapy (rAdenovirus) – 700 Sq. M. – 7,000 patients/yr**
- **Autologous Epithelial Cells – 1,000 Sq. M. – 50 to 200 (typ.)/yr**

Very Broad Range Of Outputs!

Output Variability Factors

- **Cell Line/Strain Variation/Initial Virus Titer**
- **Cell Culture Manipulations**
- **Variable Process Durations**
- **Complexity Of Purification Process**
- **Scalability of Purification Steps (OK For Bench Scale - But…?)**
- **Slow And Expensive Quality Controls/Release Testing**
- **Greatly Varying Therapeutic (Dosage) Requirements**

Perversity Reigns!

Thank you

Stephen Fitzpatrick, Ph.D, C.Eng
Senior Vice President
stephen.fitzpatrick@dpsgroupglobal.com
+1-508-861-3701 office