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Patricia NOGUIEZ-HELLIN, Généthon, France
&
Otto MERTEN, Généthon, France

Approche industrielle de Généthon pour les produits de thérapie génique

Industrial approach of Genethon for gene therapy products.

Abstract

Il y a quelques années, la complexité des procédés de bioproduction ainsi que la nécessité de manipuler des organismes génétiquement modifiés ont incité Généthon à mettre en service une structure opérationnelle de production pharmaceutique de biomédicaments et à développer le savoir-faire nécessaire.

Le développement des procédés de production, dont les procédés en aval et en amont, le transfert de technologies pour respecter les normes de BPF pharmaceutiques, la validation du remplissage aseptique et le contrôle des produits intermédiaires et finaux sont autant d'étapes nécessaires qu'il faut valider. . A ce jour, Généthon a développé des protocoles de production principalement pour les vecteurs lentiviraux et à virus adéno-associés.

Notre capacité de production actuelle est suffisante pour produire les quantités requises pour un essai clinique de Phase 1.

Cependant, pour produire des lots de vecteurs en quantité suffisante pour des essais cliniques de Phase II ou destinés à la commercialisation, il est indispensable d'établir des procédés de fabrication à grande échelle. Généthon a commencé l'industrialisation de ce type de procédés de production et prévoit d'avoir une nouvelle unité de production pharmaceutique à l'échelle industrielle opérationnelle en 2010.

Manufacturing processes complexity in addition with genetically modified organisms manipulation entail for biodrugs production a pharmaceutical structure as well as a know-how that Genethon has implemented many years ago.

Production processes development including upstream and downstream processes, technological transfers to pharmaceutical GMP norms, aseptic filling validation, in process and final products controls are as many necessary steps that must be validated. Genethon has today developed manufacturing protocols mainly for the production of AAV and lentiviral vectors.

Our capacity today is sufficient to meet the production of quantities required for phase 1 clinical trial approach.

However, to produce the vectors lot quantity necessary for phase II clinical studies or for marketing, it is indispensable to established large scale manufacturing processes.

Genethon has started the industrialization of such production processes and plans to have in 2010 a new pharmaceutical industrial production site.

Contact :

Patricia NOGUIEZ-HELLIN, PhD
Head of ETGC (GMP manufacturing sites), Qualified Person
Généthon,
1 bis rue de l'Internationale, BP60
91002 EVRY, cedex
Tel: + 33 (0)1 60 91 02 11
Fax: + 33 (0)1 60 91 02 39

**Biographie**

Depuis 2005, Patricia Nogueiez-Hellin (titulaire d'un doctorat) est à la fois responsable des sites de production pharmaceutique de Généthon (l'ETGC ou Etablissement de Thérapie Génique et Cellulaire) et la personne qualifiée pour, dans un contexte européen, autoriser la libération des lots cliniques produits.

En tant que membre de la Commission Thérapie Génique et Cellulaire de l'AFSSAPS depuis 2003, Patricia apporte son expertise dans le domaine de la thérapie génique et cellulaire.

Biography

Since 2005, Patricia Nogueiez-Hellin (PhD) is Head of both pharmaceutical production sites of Généthon (Gene and Cell Therapy Establishment or ETGC) and qualified person (European context) for pharmaceutical release of produced clinical batches.

Member of Afssaps (French Agency for Sanitary Safety of Health Products) Gene and Cell Therapy Commission since 2003, Patricia brings her expertise in the field of Gene and Cell Therapy.

Contact :**Otto Merten**

Head of the Department of Bioprocess Development
Généthon
1 bis rue de l'Internationale, BP60
91002 EVRY, cedex
Tel: + 33 (0)1 69 47 25 90

**Biography**

Otto-Wilhelm Merten has a degree in biotechnology and is the head of the department of bioprocess development of Généthon, having published more than 30 papers in refereed international journals. He has a large scientific experience gained during his stays at the Inst. Pasteur (Paris/F) as well as at the Sandoz Research Inst. (Vienna/A). Over the last 20 years, he has dealt with the development and optimization of serum-free media for the cultivation of various cell lines (hybridomas, Vero, BHK 21, MDCK, ...) and the production of different biologicals (monoclonal antibodies, various viruses). In addition, he was involved in the development of processes for the production of viruses for vaccine purposes (influenza, polio). During the last years he is involved in the development of production and purification processes for viral vectors. He is assistant professor at the Institute for Applied Microbiology at the University of Natural Resources and Applied Life Sciences in Vienna/A and gives university courses there as well as at the Universities of Creteil/F and Bobigny/F. Several students have done their Diploma and Doctorat thesis in his lab. He was co-organizer of two EU supported events, the EuroLabCourses (www.vecteurotrain.org) which were organized in Barcelona and Evry, in February and June, 2004, respectively, and he was participant in different EU sponsored projects. Between 2001 and 2005 he was the chairman of the executive committee of the European Society of Animal Cell Technology.