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## **BCG Regenerative Medicine Expertise**

Consultants from the Biologics Consulting Group (BCG) are actively involved with both academic and industry clients who are developing regenerative medicine products. These novel medical products can consist of stem cells from a variety of sources (i.e. embryonic, adult, allogeneic or autologous) and often contain genetically modified cells and/or utilize biomaterials to provide structure and facilitate delivery into patients. In some cases, regenerative medicine products are intended to integrate into the patient's own body or stimulate reservoirs of stem cells within the patient to become active and repair, replace, restore or regenerate the diseased tissue or organ.

Working with BCG's consultants from the early stages of development can significantly shorten the time it takes to reach the clinic. Our experience, gained from working at the FDA and in industry, allows us to quickly distinguish 'must do' from 'nice to know' activities. We are also proactive in helping you anticipate the key product development steps you will need to undertake to achieve commercial success.

If your regenerative medicine product contains growth factors, cells, genes, biomaterials, or a combination of these, BCG's consultants can undoubtedly help.

Below are some examples illustrating our experience with regenerative medicine products.

### **Stem Cells**

#### ***Human Embryonic Stem Cells (hESCs)***

BCG's consultants have assisted multiple academic and industry entities, both US-based and international, working to create new medical therapies derived from human embryonic stem cells (hESCs). Our advice has been key in defining the regulatory pathway for specific hESC-derived therapies, including identifying the key manufacturing issues, non-clinical safety testing requirements and appropriate clinical protocol designs for first in human studies.

We have represented clients in multiple meetings with the FDA to define requirements specific to a given therapy

#### ***Umbilical Cord Blood***

BCG's consultants have worked with numerous private and public cord blood banks throughout the U.S. We have conducted facility audits for compliance with good tissue practices (GTPs) and/or good manufacturing practices (GMPs). We have also worked with clients who are using cord blood as a starting material for novel cell-based therapies.

We are actively working with a number of cord blood banks to identify and address requirements for submission of a biologics license application (BLA).

### ***Adult Stem Cells***

BCG's consultants have extensive experience with a wide variety of adult stem and progenitor cell therapies from a variety of tissue sources.

We have worked with clients developing therapies across a broad spectrum of clinical applications. A few examples include:

- Wound Healing
- Cardiovascular Disease
- Neurological Indications (such as Stroke, De-Myelinating Diseases)
- Diabetes
- Hematological Malignancies

BCG consultants have worked with clients in establishing appropriate product release specifications, identifying contract manufacturing organizations (CMO), designing appropriate preclinical safety studies and developing first in human clinical protocols.

Related activities have included conducting quality audits for compliance with GTPs, GMPs, GLP and GCPs.

### **Gene Therapy**

We have assisted multiple academic and industry clients from around the world in the development of gene-based therapies. BCG consultants have expertise with multiple vector systems including non-viral and virus (DNA and RNA)-based vectors. Our clients include groups developing both in vivo and ex vivo gene therapies, using a variety of cells types including stem cells and somatic cells.

BCG consultants have had key input in the design of preclinical safety studies for gene-based therapies, taking into consideration the unique issues related to these products, including species and cell-type specificity and biodistribution.

BCG has also worked to solve assay and product development challenges related to gene therapy products including:

- Vector/modified cell derivation and verification
- Cell substrate and Viral Seed qualification
- In-process testing challenges
- Potency assay design
- Lot release testing
- Process development/qualification
- Stability studies
- Scale-up and comparability

Gene-based therapies have additional oversight, requiring protocol review by the NIH/RAC. BCG consultants have had first hand experience with both the FDA and NIH/RAC regulatory processes and have assisted clients in the preparation of submissions to both agencies, as well as in attendance at key agency meetings.

In addition we have conducted numerous audits of vector and cell production facilities to assess GMP and GTP compliance.

## **Biologic-Device Combination Products (Tissue Engineering)**

Tissue engineered constructs consisting of combinations of cells and natural or synthetic biomaterials require a unique skill set for efficient and successful product development.

BCG consultants with both FDA [Center for Biologics \(CBER\)](#) and [Center for Devices \(CDRH\)](#) experience will work together as a team to help you clearly define the applicable regulatory requirements and develop a focused product development strategy that will allow you to reach the clinic sooner. Examples of combination products BCG has worked on include:

- Recombinant cytokines or growth factors combined with carrier biomaterials
- Autologous cells seeded onto synthetic biomaterials to create a neo-organ
- Autologous or Allogeneic cells seeded onto natural or synthetic biomaterials for wound healing or to create conduits
- Allogeneic cells seeded into a medical device to create a novel extra-corporeal therapy
- Encapsulation of human or xenogeneic cells for various clinical indications

## **BCG's Regenerative Medicine Experts**

Below is a listing of the BCG consultants actively involved in assisting clients who are developing medical technologies used in regenerative medicine. We are here to help. Contact any of the consultants listed below to ask a question or discuss how we can assist you. Depending upon your needs, we can identify other BCG preclinical, clinical and facility consultants with expertise in this area. For further information about the background and qualifications of each consultant simply click on his or her name.

[Ellen Areman, MS, SBB](http://www.biologicsconsulting.com/cvfiles/html/areman.html) <<http://www.biologicsconsulting.com/cvfiles/html/areman.html>>

Expertise with Cell Therapy Products, Cord Blood Banking, and Cell-Processing Devices

[Angela Blackwell, MS](http://www.biologicsconsulting.com/cvfiles/html/blackwell.html) <<http://www.biologicsconsulting.com/cvfiles/html/blackwell.html>>

Expertise with Combination Products — Cell products + Medical device

[Andra Miller, PhD](http://www.biologicsconsulting.com/cvfiles/html/miller.html) <<http://www.biologicsconsulting.com/cvfiles/html/miller.html>>

Expertise Cell & Gene Therapy products

[Stuart Portnoy, MD](http://www.biologicsconsulting.com/cvfiles/html/portnoy.html) <<http://www.biologicsconsulting.com/cvfiles/html/portnoy.html>>

Expertise with Combination Products — CTGT products + device delivery systems

[Darin Weber, PhD](http://www.biologicsconsulting.com/cvfiles/html/weber.html) <<http://www.biologicsconsulting.com/cvfiles/html/weber.html>>

Expertise in regulatory strategy and product development for cells, tissues and regenerative medicine products

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## Understanding and Implementing Good Tissue Practices

article in RAPS Focus by Darin Weber, PhD, Senior Consultant

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Many of the requirements of the Good Tissue Practice (GTP) regulation will be familiar to those who work under Good Manufacturing Practice (GMP) or Quality Systems Regulations (QSRs). However, it is important to recognize that GTPs do have some unique requirements not found within existing GMP/QSR regulations. Thus, to ensure compliance with GTPs, a systematic assessment of current quality systems is essential to identify and fill any gaps. This article provides an overview of GTPs and offers considerations for assessing the state of compliance with GTP requirements.

### What are GTPs?

GTPs are a comprehensive set of regulations enacted by the US Food and Drug Administration (FDA) to cover human cells, tissues or cellular or tissue-based products (HCT/Ps) intended for implantation, transplantation, infusion or transfer into a human recipient. These regulations govern the methods used in, and the facilities and controls used for, the manufacture of HCT/Ps. The primary intent of GTPs is to ensure that HCT/Ps are manufactured in a manner designed to prevent the introduction, transmission and spread of communicable disease. In some cases, an HCT/P may be subject only to GTP requirements. In other cases, e.g., when an HCT/P is the starting material for creating novel cell- and tissue-based products, GTPs and additional regulatory requirements, such as GMPs, biological product standards and/or the medical device QSR, will be applicable. The criteria governing the specific level of regulatory oversight are described in 21 CFR 1271.10 and have been reviewed elsewhere. Figure 1 depicts how GTPs apply to different regulatory pathways for an HCT/P.

[Continue Reading](http://www.biologicsconsulting.com/articles/GTPs_Weber.pdf) <[http://www.biologicsconsulting.com/articles/GTPs\\_Weber.pdf](http://www.biologicsconsulting.com/articles/GTPs_Weber.pdf)>

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## 15 Years Providing Regulatory Expertise



Over the last 15 years, BCG has grown from a 5 person mom-and-pop shop biologics focused consulting group to a 50-person small business with expertise in biologics, drugs and devices and personnel from the Agency (CBER, CDER and CDRH) as well as industry. Thankfully for our clients, we maintain the responsiveness and intimacy that you would expect of a small group and believe this is our greatest asset.

Today [Biologics Consulting Group, Inc.](http://www.biologicsconsulting.com) specializes not only in the preparation and review of CBER regulatory applications (INDs and BLAs) as well as the inspection/audit of biologics manufacturing facilities but also in similar regulatory activities for drugs (CDER), device (CDRH) and combination products. Because of our familiarity with FDA expectations we have an excellent reputation at the Agency for filing high-quality, easily reviewable applications.

## PRODUCT EXPERIENCE

- Allergenic Products
- Antitoxins, antivenins and venoms
- Blood Products
- [Cell and Tissue Products](http://www.biologicsconsulting.com/Brochure_Cell,%20Tissue%20and%20Gene%20Therapy)  
<[http://www.biologicsconsulting.com/Brochure\\_Cell,%20Tissue%20and%20Gene%20Therapy](http://www.biologicsconsulting.com/Brochure_Cell,%20Tissue%20and%20Gene%20Therapy)>
- Combination Products (Medical Device/Biologic)
- Drugs (Small molecules)
- [Gene Therapy](http://www.biologicsconsulting.com/Brochure_Cell,%20Tissue%20and%20Gene%20Therapy)  
<[http://www.biologicsconsulting.com/Brochure\\_Cell,%20Tissue%20and%20Gene%20Therapy](http://www.biologicsconsulting.com/Brochure_Cell,%20Tissue%20and%20Gene%20Therapy)>
- In-vivo Diagnostics
- [Medical Devices](http://www.biologicsconsulting.com/Brochures_Devices.pdf) <[http://www.biologicsconsulting.com/Brochures\\_Devices.pdf](http://www.biologicsconsulting.com/Brochures_Devices.pdf)>
- Therapeutic Proteins
- [Vaccines](http://www.biologicsconsulting.com/Brochure_Influenza.pdf) <[http://www.biologicsconsulting.com/Brochure\\_Influenza.pdf](http://www.biologicsconsulting.com/Brochure_Influenza.pdf)>

## SERVICE EXPERIENCE

<<http://www.bcg-usa.com/services/services.php>>

- [Pre-IND/IND, Pre-BLA, BLA, MF Support](#) including [electronic submissions](#)
- [Clinical Development Support](#)
- [Pharmacology/Toxicology Support](#)
- [Facilities Support](#)
- [Process Validation](#)

- [Product Development](#)
- [Program Management](#)
- [US Agent for Non-US and US Manufacturers](#)
- Vendor/ Contract Lab Audits
- Orphan Drug Applications
- Due Diligence audits
- [Training Courses](#)
- Biostatistical Analysis

## **View BCG's Expertise Listing**

(<http://www.bcg-usa.com/staff/expertise.php>)

### **Upcoming BCG Presentations & Conference Appearances**

(For complete list and speakers... <http://www.bcg-usa.com/appearances/appear.php>)

BCG consultants attend numerous conferences every year during which they are available to provide company information or consulting expertise. Below are some conferences that BCG consultants will be attending in the near future. Please feel free to contact the attending consultant either before, or during the conference for company information or to discuss a business opportunity.

<b>Date</b>	<b>Sponsoring Organization</b>	<b>Conference and Presentation Title</b>	<b>BCG Attendee(s)/ Speaker (s)</b>	<b>Location</b>
Sept. 8-12, 2008	PDA/FDA	<a href="#">2008 PDA/FDA Joint Regulatory Conference</a>	<a href="#">Nadine Ritter, Ph.D.</a>	Washington, DC
Sept. 14-17, 2008	<a href="#">Regulatory Affairs Professional Society (RAPS)</a>	<a href="#">Annual Conference &amp; Exhibition</a> "Follow-on Biologics, (Biosimilars)" (Subbarao)	Speakers <a href="#">Nanda Subbaro, Ph.D.</a>  <a href="#">Lorianne Baranauskas</a> <a href="#">John Jessop, PhD</a> <a href="#">Reggie Neal</a> <a href="#">Kelly Reich, MS</a> <a href="#">Michael Trapani, MS, MBA</a> <a href="#">Holli Vaughan, MS, RAC</a> <a href="#">Keith Wells, Ph.D.</a>	Boston, MA
Sept. 24, 2008	Georgia Bio	<a href="#">2008 Georgia Life Sciences Summit</a>	<a href="#">Eugene Johnston, CQE, CQA</a> <a href="#">Jim Kenimer, PhD</a>	Atlanta, GA
Sept 28-Oct 4, 2008		ISO TC 106 Dental Devices Annual Meeting	<a href="#">Angela Blackwell, MS</a>	Göteborg, Sweden
Sept. 30, 2008	<a href="#">Benastrum</a>	<a href="#">The Significance of Quality Systems</a>	<a href="#">Eugene Johnston, CQE, CQA (speaker)</a>	Webinar

Oct 4-7, 2008	<a href="#">AABB</a> (American Association of Blood Banks)	<a href="#">Annual Meeting and TXPO</a>	<a href="#">Ellen M. Areman, MS, SBB</a> ( <b>session chair</b> )	Montreal, Canada
Oct 9-10, 2008	International Pharmaceutical Academy	Conducting Failure Investigations	<a href="#">Eugene Johnston, CQE, CQA</a> ( <b>speaker</b> )	Toronto, Canada
Oct. 15-17, 2008	Bio-Japan	<a href="#">Bio-Japan</a>	<a href="#">Jim Kenimer, PhD</a> <a href="#">T.W. Tanaka, PhD</a> <a href="#">Shin-ichi Kamachi, PhD</a> <a href="#">Masamichi Gotoh</a>	Yokohama, Japan
Oct 22-23, 2008	<a href="#">IIR</a>	<a href="#">Stability Testing for Biotechnology Products</a>	<a href="#">Nadine Ritter, Ph.D.</a> ( <b>speaker</b> )	London, UK
Nov 9-12, 2008	<a href="#">ACT (American College of Toxicology)</a>	29th Annual Meeting Introduction of Biologics (Hartsough)	<a href="#">John Jessop, Ph.D., MPH</a> <a href="#">Melanie Hartsough, Ph.D.</a> ( <b>speaker</b> ) <a href="#">David J. Pepperl, Ph.D.</a>	Tuscon, AZ
Nov 10-11, 2008	<a href="#">PDA (Parenteral Drug Association)</a>	<a href="#">Development and Regulation of Clinical Trial supplies</a>	<a href="#">Eugene Johnston, CQE, CQA</a> ( <b>speaker</b> )	Boston, MA
Nov 18-21, 2008	ASTM	November Meeting week Division IV Tissue Engineered Medical Products	<a href="#">Angela Blackwell, MS</a>	Miami Beach, FL

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