CTI Clinical Trial and Consulting Services

Volume 10, Issue 8 August 2012 Newsletter

CTI Clinical Trial and Consulting Services is a repeat winner of "Best Places to Work" in Greater Cincinnati

We are recruiting for CRAs in the US, UK, France, Belgium and the Netherlands!

Upcoming Medical Meetings CTI will be Attending ...

Galectin Science and Therapeutic Applications Symposium
Boston, MA
September 17 – 19

Stem Cells USA & Regenerative Medicine Congress
Boston, MA
September 20 – 21

If you are interested in scheduling a meeting with CTI at one of these events, please contact Nick

Orphan Drugs

Orphan drugs are defined as medicinal products for diagnosing, preventing, or treating rare medical conditions that are life-threatening or very serious. Many countries offer financial incentives for sponsors to develop drugs for orphan indications because the development costs would exceed expected sales revenue in these small patient populations under normal market conditions.

The incentives offered vary from country to country, and can be of substantial value. In the US, tax credits are given for the cost of drug development in orphan diseases. The FDA waives the marketing application fee for orphan drugs (currently close to $2 Million), grants 7 years of marketing exclusivity after approval, assists in clinical study design, and administers a program of research grants to defray the cost of clinical testing. A company that develops a drug for a pediatric rare disease receives a priority review voucher that can be used to obtain priority review for any future product that would otherwise receive a standard review.

In Europe, incentives for orphan drugs include 10 years of marketing exclusivity, scientific advice on protocol development, fee reductions for marketing applications and other fee-based regulatory activities, and grant money to help fund research.

In some clinical areas, like solid organ transplants, the size of the patient population allows all new therapies to qualify for orphan status if they target an unmet medical need or provide a clinically meaningful improvement over existing therapies. Developing a drug with potential benefit in several diseases for an orphan indication first can be a good commercial strategy if the clinical science fits this model. **A successful orphan program can get a new drug on the market more quickly, at a lower cost, while programs in larger populations are planned to run concurrently or after the orphan program.**

In addition to identifying the unmet medical need or expected clinical benefit, an orphan drug designation request must include

Schatzman at 513-598-9290 or via email at nschatzman@ctifacts.com

---

Employee Update

Please welcome the newest addition to CTI:

Ryan Bodle – Senior Accountant

Congratulations to the following CTI employees recently promoted:

Mike Montani – Manager, Business Development Operations

Kirsten Cooney – Associate Study Manager

Quick Links…

Our Website

Email

Join Our Mailing List!

documentation of the size of the target population. The threshold for a "rare" disease or condition is set at no more than 200,000 persons in the US and no more than 5 in 10,000 persons in the countries of the European Union. In Europe this information must be provided for each country individually, so it is not necessarily an easily accomplished task. **CTI can perform the research needed to document that the size of the patient population meets requirements, wherever the sponsor plans to file.**

Applications for orphan drug designation can be filed at any time before a marketing application is submitted, though **CTI recommends submission early in a product's development so that the sponsor can take full advantage of the incentives offered.** A joint US-European orphan drug designation request form is available, so that a single application can be prepared for use with both regulatory agencies.

CTI regulatory consultants have extensive experience writing and filing orphan drug designation requests on behalf of our clients. CTI consultants can write all sections of the request, adapting existing sponsor documents, adding our independent research where needed, and providing the required regulatory formats. We can submit your orphan drug designation request and follow up as needed with the regulatory agencies to ensure a smooth approval process.

To learn more about Orphan Drugs and how CTI may be of assistance, please contact us.

Kathryn Wekselman, PhD RN
Director, Regulatory and Scientific Affairs
CTI Clinical Trial and Consulting Services
Phone: 513.598.9290
Fax: 513.598.3426
Email: kwekselman@ctifacts.com
Website: www.ctifacts.com