Fast-tracking rare disease drug candidates' approval

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Aspiring new drugs face long and stringent tests on safety and effectiveness before making it to market. And rightly so. But giving drugs special designations that bring with them the right incentives can help bring them to patient. One such drug is OPN-305, a drug candidate that belongs to the family of monoclonal antibodies and might counter inflammation in transplanted kidneys. It has been developed by Opsona Therapeutics a biopharmaceutical company located in Dublin, Ireland.

This drug candidate is now undergoing Phase II clinical trials as part of the EU-funded project MABSOT, due to be completed in 2014. OPN-305 received orphan drug designation for prevention of solid organ transplantation from the European Medicines Agency and the US Food and Drug Agency (FDA). Incentives, attached to such designation, include scientific advice. And, once a drug candidate reaches the market, it also brings market exclusivity. Project coordinator Mary Reilly, who is also vice president of pharmaceutical development and operations at Opsona Therapeutics, talks to youris.com about the benefits of obtaining orphan drug status.

Under what conditions can a drug candidate be classed as an orphan drug and what does it imply?

This is basically a drug for a rare disease. So if it affects not more than five per 10,000 Europeans, we can apply during development for orphan status. We have to show significant benefit over existing treatment. The

designation does not guarantee that you are going to get market authorisation or anything like that, but it is positive. There are certainly benefits in terms of getting scientific advice from the European Medicines Agency.

Is scientific advice helpful when you are a start-up?

Absolutely, it is hugely helpful. A lot of people have mixed views about whether you should approach regulatory authorities early or late in the development process. Our view has always been to approach the authorities as early as possible and we found that to be very beneficial.

What benefits can an orphan drug designation bring?

From a regulatory perspective it means that you can get accelerated approval. So you can move through the development process quicker. There are no fees associated with the application. And you get benefits like free scientific advice and assistance. Also, if our product is approved we can get substantial benefits over other products. You can get market exclusivity from the European Medicines Agency for ten years and seven years from the FDA in the USA. So no one can submit a market authorisation application for that specific indication during that period, unless they show significant benefit over your product.

If everything goes well with your drug candidate OPN-305, what other type of transplantations might it be useful for?

We already have orphan designation for solid organ transplantation. So we could do lung transplantation, heart transplantation, or pancreatic transplantation; lots of different types. It also has applicability for other diseases, including cancer and rheumatoid arthritis.

Will orphan drug designation for your compound help move this drug candidate forward for other conditions?

Not necessarily. Because you would have to generate the support of preclinical data [i.e. data gathered on animals before it is tested in humans] in those areas in order to justify going into them. For now, we decided to pursue the acute indication of renal transplantation as the first indication. But we will pursue other indications for the antibody as well. Also, you can apply for orphan drug designation multiple times for the one drug candidate. So long as you meet the criteria required.

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