Patenting and treatments for rare disorders

Key points

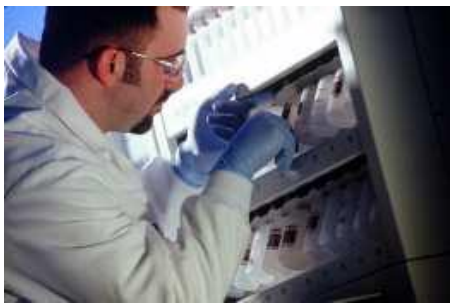
- Patents are critical to R&D and bringing products to market. The investment required to bring a product to patients is substantial – over $1 billion. This type of investment is unlikely to occur if companies cannot gain a return through patent protection.
- Genzyme's treatments for rare genetic disorders are directed towards harnessing naturally-occurring biological and genetic material to replace or repair missing or defective molecules in patients with these conditions.
- Biological patents do not confer ownership over natural material, only molecules in their isolated form. They also have to be a directed towards a *specific purpose*, such as treating a particular disorder.
- By banning all patenting of genetic and biological material, the *Patent Amendment (Human Genes and Biological Materials) Bill 2010* (Private Member's Bill) would undermine the ability of Australians to participate in and benefit from this entire area of medical science.

How are treatments for rare diseases developed?

Genzyme has a particular focus on enzyme replacement therapies for rare genetic conditions such as Lysosomal Storage Disorders (LSDs). LSDs are extremely rare, typically affecting fewer than 100 people in Australia. In LSDs, patients are born without an enzyme that is needed to break down waste products in cells. This leads to complications in multiple organs and these diseases are often life-threatening at an early age.

Genzyme has developed several treatments for LSDs, where the naturally occurring enzyme is replaced by a synthetic version, allowing patients to stabilise or recover from the disease. Patients have to receive regular infusions of enzyme for the rest of their lives. These products have been found to be life-saving in many patients and to also significantly improve the quality of life.

To develop treatments, the naturally occurring enzyme has to be identified, isolated and reproduced in a therapeutic form. Creating a drug that is proven to be safe and efficacious is not a simple process and requires significant research, intellectual property and investment. Patent protection is the first step in this pathway.



Consequences of this legislation

The Bill seeks to amend the *Patents Act 1900* (Cth) by providing that "biological materials including their components and derivatives, whether isolated or purified or not and however made, which are identical or substantially identical to such materials as they exist in nature" are not patentable inventions. Denying patent protection to biological products could have serious negative consequences for Australia. Without appropriate protection, companies like Genzyme would be disincentivised to make the enormous investments required to bring products to market, potentially denying Australians with rare diseases access to life saving therapies.

Genzyme is currently working on gene therapies for a range of diseases such as Parkinson's disease. If the Bill is enacted into legislation, it would also lessen the attractiveness of Australia as a destination for clinical trials, compromising early access by Australians to these therapies.

Case study – Australian discoveries in rare disorders

The MPS (mucopolysaccharidosis) diseases are rare genetic conditions that can lead to complications with the heart, bones, joints, respiratory system and central nervous system. Treatments for MPS I and MPS II, both provided in Australia by Genzyme, started with research conducted in Adelaide at the Women's and Children's Hospital in the 1990s. The Hospital holds patents to both alpha-L-iduronidase and iduronate 2-sulfatase – the natural enzymes missing in patients with MPS I and MPS II respectively. These patents identified the potential for using synthetic enzymes to replace the missing molecules and treat these diseases.

Development on these products was then driven by biotechnology companies who refined the manufacture and delivery of these enzymes therapies. This involved significant R&D, including clinical trials in MPS patients to demonstrate that treatment works and improves patient outcomes.

As a result of this investment and development, Australian children and adults with MPS I and MPS II are now receiving life-saving treatment, and researchers in Adelaide are receiving royalties that fund continued world-class research.

Without patent protection for the natural enzymes that are missing in the MPSs and the genetic codes needed to make them, biotechnology companies would have found development risky and may not have invested in taking these products to market. Companies would have had to take on significant development risks and expenses without any assurance of being able to make a return on that investment.

About Genzyme

Since its founding in 1981, Genzyme has grown from a small start-up to a diversified enterprise with a focus on meeting unmet medical needs and identifying breakthrough medicines to treat these conditions. Genzyme's products and services are focused on rare inherited disorders, kidney disease, orthopaedics, cancer, transplant and immune disease, and diagnostic testing. Genzyme has been operating in Australia since 1994. At present, Genzyme Australasia has nearly 60 employees. Genzyme is part of the sanofi-aventis Group.

About sanofi-aventis

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