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Institut d'Investigació Biomèdica de Bellvitge

IDIBELL Technology Portfolio
Ref. 16JAP004

Drug repurposing for lupus nephritis therapy

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HIGHLIGHTS

- ✓ Less side effects than the standard of care
- ✓ Already approved for human in other medical indications (Reprofiling)
- ✓ Market with 1 approval in last 50 years

TECH STATUS

- ✓ **TRL:** Late Preclinical Studies
- ✓ **IP**: Patent Application EP17382491.3

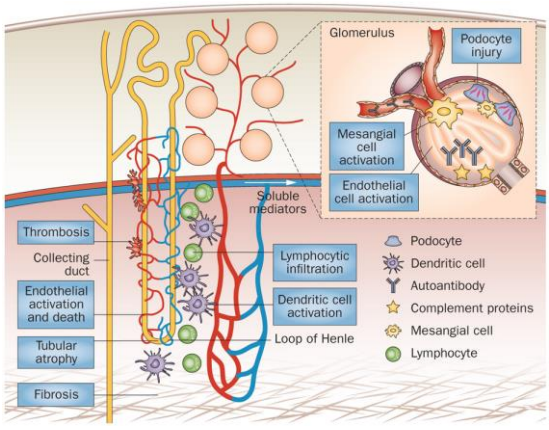
Problem to be solved

Lupus nephritis management consists of an initial (induction) phase and a maintenance (extended) phase in which steroids are used in combination with another immunosuppressive medication. Current treatments are incompletely effective and associated with substantial toxicity.

Background

Lupus nephritis is a type of kidney disease caused by systemic lupus erythematosus (SLE or lupus). Lupus is an autoimmune disease —a disorder in which the body's immune system attacks the body's own cells and organs. Up to 60% of lupus patients will develop lupus nephritis.

The introduction of corticosteroids and later, cyclophosphamide dramatically improved survival in patients with proliferative lupus nephritis, and combined administration of these agents became the standard-of-care treatment for this disease. However, treatment failures were still common and the rate of progression to end stage renal disease (ESRD) remained unacceptably high. Additionally, treatment was associated with significant morbidity. Therefore, as patient survival improved, the goals for advancing lupus nephritis treatment shifted to identifying therapies that could improve long-term renal outcomes and minimize treatment-related toxicity. Unfortunately, progress has been slow and the current approaches to the management of lupus nephritis continue to rely on high-dose corticosteroids plus a broad-spectrum immunosuppressive agent.



Technology

Our compound X is a pharmacological agent already approved for human use in other medical indications. When orally administered, compound

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X can be repurposed to prevent or attenuate the development of lupus nephritis and, eventually, other SLE manifestations, without the toxicity and side effects associated with the continued use of corticosteroids and immunosuppressant drugs.

Oral bioavailability: > 90%; biological half-life: up to 48 h; excretion: feces and urine.

Applications

The principal goal of compound X therapy in lupus nephritis is to normalize renal function or, at least, to prevent the progressive loss of renal function. Therapy differs depending on the pathologic lesion. It might also be important for the treatment of other SLE manifestations, or even other autoimmune diseases.

Technology status

In vitro assays, compound X has been shown to reduce the expression of co-stimulation and maturation markers from inflammatory monocyte-derived dendritic cells.

Moreover, in the LN murine model (LZB/LZW F1 mice), oral administration of compound X after the beginning of disease (week 30 of age) was able to significantly delay the onset and to reduce the level of proteinuria (to improve renal function) compared to non-treated LZB/LZW F1 mice. This translated to: 1) preservation of weight with age; and 2) 100% survival compared to non-treated LZB/LZW F1 mice (8% survival) at the end of the study (330 days). All these results show that the technology is ready for clinical trials.

IP status: Patent application ongoing.

Market Opportunity

The growing prevalence of lupus nephritis and awareness among patients have propelled the growth of the global lupus nephritis market, which has been segmented into four key regions: North America, Asia Pacific, Europe, and Rest of the World. Robust healthcare infrastructure and research and development activities in developed economies in North America and Europe have propelled the growth of the lupus nephritis market in these regions. The growing prevalence of the disease in North America has also augmented the market growth. It is estimated that about 1.5 million Americans suffer from lupus.

The lupus therapeutics market is dominated by generics and GlaxoSmithKline's Benlysta is the only drug that has gained marketing approval for SLE in more than 50 years. Following Benlysta's launch, patients' uptake has remained low, but due to its associated high price-tag Benlysta has managed to grow the lupus market in terms of value and is currently the top-selling drug in this market, having generated approximately $265.6m in 2014 in the seven major markets: United States, EU5 (France, Germany, Italy, Spain, UK) and Japan.

Business Opportunity

- Co-development or license agreement.

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