

New treatment for mosaic overgrowth syndromes

CLOVES – PIK3CA- Overgrowth syndromes



CONTEXT

Mosaic overgrowth syndromes (ex CLOVES syndrome) are very disabling orphan diseases. Except amputation, there is no treatment with sufficient effectiveness at the present time.

Recent research has shown a role of PI3K gene in the cell hyperproliferation process, paving the way for new treatments.

DESCRIPTION

Our lab has shown antiproliferative effects of a specific PI3K molecule on in vitro human cell cultures (blocking the passage to S phase – 39% reduction in cell growth). Those molecular effects are observed further down the metabolic pathway than what has been shown in the latest research on CLOVES syndrome.

As our molecule was more potent in vitro than BYL719 (Alpelisib - a clinically tested inhibitor of the PI3K/AKT pathway) in inhibiting the fibroblasts growth from patient, we believe that this feature gives our molecule higher specificity for treatment.

We filed a patent for this molecule for its use on mosaic overgrowth syndromes, seborrheic keratosis and cutaneous vascular malformations related to PI3K gene.

Since there is no cure for overgrowth syndromes related to PI3K gene, our discovery will probably open a new treatment alternative for patient care.

COMPETITIVE ADVANTAGES

- **Mode of action with higher specificity**
⇒ May lead to less side effects (vs current medications)
- **New treatment option for patients**
⇒ No equivalent treatment available on market



Markets and applications

Pharmaceuticals:

- ❖ Overgrowth syndromes related to PIK3CA gene mutations
- ❖ Seborrheic keratosis and cutaneous vascular malformations related to PI3K gene



Development stage

TRL 4 : In vitro test on human cells



Intellectual property

European patent application filed on October, 31st 2017



Target partnership

Patent licensing

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