RARE DISEASE - MITOCHONDRIAL

Therapeutic use of redox cyclers in diseases linked to mitochondrial respiratory chain dysfunction

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What it is needed for?

Mitochondrial disorders are debilitating and life-threatening diseases that still **lack effective treatment**. They are due to genetic defects affecting oxidative phosphorylation, thus mitochondrial fitness and ATP production.

Patients with complex III dysfunction develop progressive neurological impairment, myopathy and meet an early death. Currently, no treatment for such pathologies exist.

We have developed a **pharmaceutical strategy to "replace" the defective complex III**. We found that redox cyclers (compounds undergoing reduction to form radical species that can then react with oxygen, thus regenerating the parent molecule), such as pyocyanin, can recover the mitochondrial function in patient-derived cells and ameliorate the pathological phenotypes animal models of complex III deficiency.

See also Peruzzo et al., Nat Commun (2021) .

Advantages

- No treatment for mitochondrial diseases linked to complex III dysfunction exist;
- A treatment for rare diseases, thus eligible for Orphan drug designation;
- Pyocyanin is a small molecule of bacterial origin able to penetrate biological membranes.

Applications

A drug for treatment of diseases due to the dysfunction of mitochondrial electron transfer chain complexes, such as complex III.

TRL scale

1 2 3 4 **5** 6 7 8 9

Health and biomedical - Pharmaceuticals

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