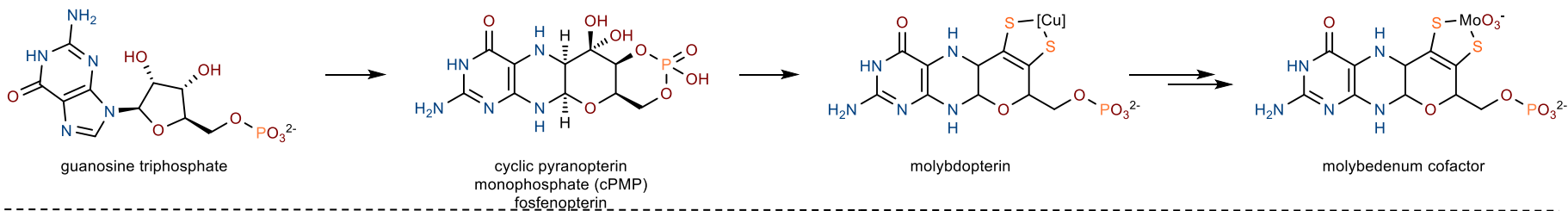


- Discovered in the University of Braunschweig and the University of Cologne
- FDA approved in February 2021
 - Granted breakthrough therapy, orphan drug status
- Used to reduce the mortality rate for patients with Molybdenum cofactor deficiency (MoCD)
 - Mean survival in untreated patients is 36 months
 - Survival rate increases from 55% to 84%
 - 0.9 mg/kg once daily – after three months
- Leads to possible phototoxicity in patients



Molybdenum cofactor biosynthesis



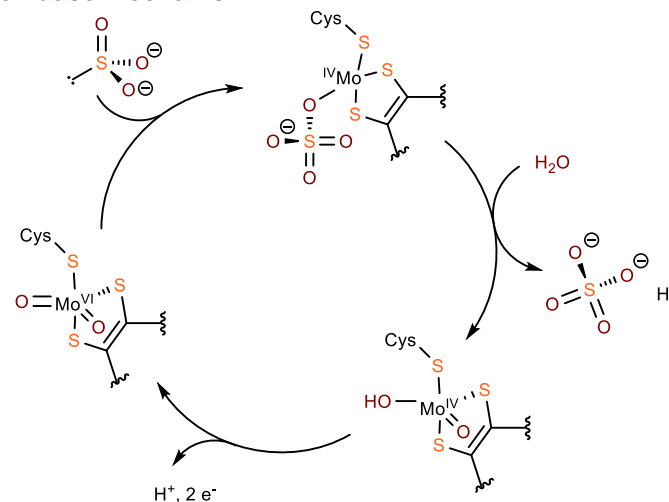
MoCD

- Deficiency is an extremely rare metabolic disease known as MoCD
- Severe neurological damage, dysmorphic facial features, progressive cerebral palsy, seizures, early death
- Approx. 100 patients found to have MoCD since 2010 – believed to be underdiagnosed

Mechanism of action

- Replaces an intermediate in the synthesis of molybdopterin cofactors
- Cofactors found as ligands in molybdenum and tungsten containing enzymes
 - Caused by absence of activity of sulfite oxidase
 - Critical in the metabolism of sulfur containing compounds

Sulfite oxidase mechanism



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