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Crigler Najjar Syndrome - Pipeline Review - 2019

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Abstracts

Firstview Insight's Crigler Najjar Syndrome - Pipeline Review-2019 provides an overview of the pipeline landscape of Crigler Najjar Syndrome It provides comprehensive insights of all the clinical and non-clinical therapeutics in development with detailed description about the collaborations; deals; designations; patent information etc.These reports encourage the clients in distinguishing the upcoming and existing competitors in their separate therapeutic spaces. The report provides detailed description of the competitor profiles with key milestones and evidence along with analysis by mechanism of action; route of administration; molecule type; stage of development. Information obtained from multiple sources will be used to triangulate and update the profiles. The report also provides key events in the last year related to the indication. This report provides detailed analysis of all the products along with the companies involved.

Crigler–Najjar syndrome is a rare inherited disorder affecting the metabolism of bilirubin, a chemical formed from the breakdown of the heme in red blood cells. The disorder results in a form of nonhemolytic jaundice, which results in high levels of unconjugated bilirubin and often leads to brain damage in infants. The disorder is inherited in an autosomal recessive manner.

This syndrome is divided into types I and II, with the latter sometimes called Arias syndrome. These two types, along with Gilbert's syndrome, Dubin–Johnson syndrome, and Rotor syndrome, make up the five known hereditary defects in bilirubin metabolism. Unlike Gilbert's syndrome, only a few causes of CNS are know It is caused by abnormalities in the gene coding for uridine diphosphogluconurate glucuronosyltransferase (UGT1A1). UGT1A1 normally catalyzes the conjugation of bilirubin and glucuronic acid within hepatocytes. Conjugated bilirubin is more water soluble and is excreted in bile.Type I

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This is a very rare disease (estimated at 0.6–1.0 per million live births), and consanguinity increases the risk of this condition (other rare diseases may be present). Inheritance is autosomal recessive.

Intense jaundice appears in the first days of life and persists thereafter. Type 1 is characterised by a serum bilirubin usually above 345 µmol/L [20 mg/dL] (range 310–755 µmol/L [18–44 mg/dL]) (whereas the reference range for total bilirubin is 2–14 ?mol/L [0.1-0.8 mg/dL]).

No UDP glucuronosyltransferase 1-A1 expression can be detected in the liver tissue. Hence, there is no response to treatment with phenobarbital,[1] which causes CYP450 enzyme induction. Most patients (type IA) have a mutation in one of the common exons (2 to 5), and have difficulties conjugating several additional substrates (several drugs and xenobiotics). A smaller percentage of patients (type IB) have mutations limited to the bilirubin-specific A1 exon; their conjugation defect is mostly restricted to bilirubin itself.

Before the availability of phototherapy, these children died of kernicterus (bilirubin encephalopathy) or survived until early adulthood with clear neurological impairment.

Today, therapy includes

exchange transfusions in the immediate neonatal period
12 HOURS/DAY PHOTOTHERAPY
heme oxygenase inhibitors to reduce transient worsening of hyperbilirubinemia (although the effect decreases over time)
oral calcium phosphate and carbonate to form complexes with bilirubin in the gut
liver transplantation before the onset of brain damage and before phototherapy becomes ineffective at later age
Type II
The inheritance patterns of both Crigler–Najjar syndrome types I and II are autosomal recessive.[2]
However, type II differs from type I in a number of different aspects:
Bilirubin levels are generally below 345 µmol/L [20 mg/dL] (range 100–430 µmol/L [6–24 mg/dL]; thus, overlap may sometimes occur), and some cases are only detected later in life.
Because of lower serum bilirubin, kernicterus is rare in type II.
Bile is pigmented, instead of pale in type I or dark as normal, and monoconjugates constitute the largest fraction of bile conjugates.

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UGT1A1 is present at reduced but detectable levels (typically

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