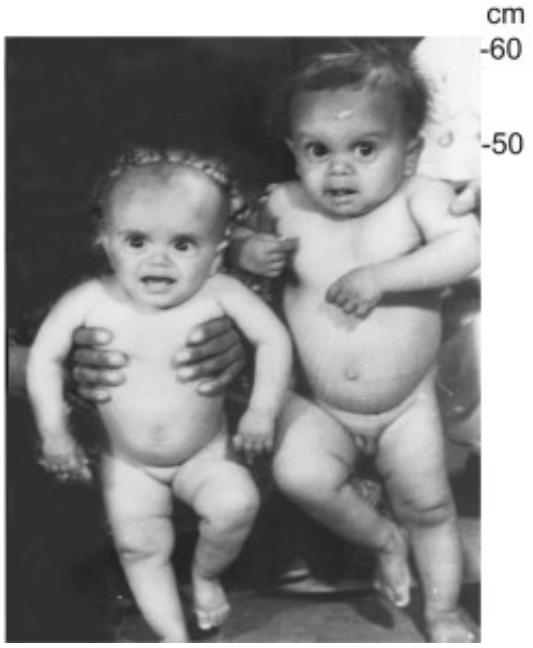
The future of Laron syndrome



Laron syndrome (LS) first described 50 years ago in 1966 is with few exceptions a genetic form of severe short stature, found in the majority of instances in patients originating from the Mediterranean, Middle East and South Asian regions. It is a hereditary disease due to marriage within the family. However there are exceptions. It is caused by a faulty mechanism of the growth hormone (GH) action due to molecular changes (mutations or deletions) in the GH-receptor (i.e. the place in the cell which transmits the GH action). Thus there is no GH activity. GH acts by initiating the creation of a hormone synthesized in the liver cell (insulin like growth factor 1=IGF-I) which induces the GH effect. Due to the molecular defect there is no IGF-I production in Laron syndrome.

Lack of IGF-I results in growth retardation, obesity, elevated blood lipids and with time even diabetes. Of great interest is our finding that Laron syndrome patients are protected from cancer even if treated.

The only treatment for this disease is daily subcutaneous injection of biosynthetic IGF-I. Early initiation of treatment accelerates the growth, in these patients, rising their quality of life. Unfortunately this drug is very expensive and therefore many patients in countries without a National Health program are not treated and often not diagnosed.

As Laron syndrome is a rare but treatable disease (grouped under orphan diseases) health authorities should advocate and provide payment for the diagnosis and treatment. In our opinion small doses of IGF-I should also be given to adult patients with Laron syndrome to strengthen their muscular and skeletal systems.

Publication

Fifty seven years of follow-up of the Israeli cohort of Laron Syndrome patients-From discovery to treatment.
Laron Z, Kauli R
Growth Horm IGF Res. 2015 Aug 18