

A

Can consensus guidelines (for management) be achieved for patients with liver Glycogen Storage Disease?

A

Interim Rankings

Patients: 71

Caregivers: joint 58

Health professionals: joint 8

B

How can existing cornstarch preparations be modified or alternative treatments be implemented that are easier to administer and/or keep blood sugar levels more stable for patients with liver Glycogen Storage Disease?

B

Interim Rankings

Patients: joint 9

Caregivers: 4

Health professionals: 4

C

How can patients with liver
Glycogen Storage Disease
achieve and/or maintain a
healthy weight throughout
life?

C

Interim Rankings

Patients: joint 3

Caregivers: 8

Health professionals: joint 33

D

How can we improve the
quality of life of patients with
liver Glycogen Storage
Disease?

D

Interim Rankings

Patients: joint 13

Caregivers: 10

Health professionals: joint 39

E

How is the (natural)
progression of liver Glycogen
Storage Disease at different
stages of life?

E

Interim Rankings

Patients: joint 5

Caregivers: 5

Health professionals: joint 39

F

How should optimal metabolic control both clinically and biochemically (like lactate, ketones and/or lipids) be achieved in liver Glycogen Storage Disease?

F

Interim Rankings

Patients: joint 20

Caregivers: joint 32

Health professionals: joint 5

G

How should sickness and emergency situations be managed for patients with liver Glycogen Storage Disease?

G

Interim Rankings

Patients: joint 9

Caregivers: 7

Health professionals: 18

H

How to manage diet regimen in relation to "before, during and after" physical exercise (sport, playing) for patients with liver Glycogen Storage Disease?

H

Interim Rankings

Patients: joint 5

Caregivers: 3

Health professionals: 14

I
How to prevent and/or treat
hormonal problems (i.e.
thyroid, menstrual cycle,
growth, diabetes, insulin
response) in patients with liver
Glycogen Storage Disease?

I

Interim Rankings

Patients: joint 5

Caregivers: joint 32

Health professionals: 45

J

How to prevent and/or treat
muscle problems in patients
with liver Glycogen Storage
Disease?

J

Interim Rankings

Patients: 2

Caregivers: joint 24

Health professionals: joint 22

K

How to prevent and/or treat
neurological problems in
patients with liver Glycogen
Storage Disease?

K

Interim Rankings

Patients: 8

Caregivers: 65

Health professionals: joint 66

L

What are the best options (for example gene therapy or enzyme replacement therapy) for achieving sufficient amount of working enzyme in patients with liver Glycogen Storage Disease?

L

Interim Rankings

Patients: joint 3

Caregivers: 6

Health professionals: joint 12

M

What are the consequences of consumption of alcohol and drugs for patients with liver Glycogen Storage Disease?

M

Interim Rankings

Patients: joint 9

Caregivers: 29

Health professionals: 46

N

What are the effects of
different kinds of Ketogenic
Diet in patients with
Glycogen Storage Disease
Type III?

N

Interim Rankings

Patients: joint 20

Caregivers: joint 48

Health professionals: 7

0

What are the long-term complications (liver, renal, gut) of a diet rich in uncooked cornstarch and/or high protein and should the diet be adjusted to prevent complications in liver Glycogen Storage Disease?

0

Interim Rankings

Patients: joint 9

Caregivers: 1

Health professionals: 3

P

What are the risks and benefits of different options for overnight treatment for patients with liver Glycogen Storage Disease and how can we maximize safety?

P

Interim Rankings

Patients: joint 48

Caregivers: joint 22

Health professionals: joint 10

Q

What are the risks and benefits of gene therapy for patients with liver Glycogen Storage Disease?

Q

Interim Rankings

Patients: 1

Caregivers: 2

Health professionals: joint 12

R

What is the best way to start dietary treatment, finding the optimal doses, and to administer the diet for patients with liver Glycogen Storage Disease?

R

Interim Rankings

Patients: joint 34

Caregivers: joint 37

Health professionals: joint 10

S

What is the needed
restriction of lactose,
fructose or saccharose in
different types of liver
Glycogen Storage Disease?

S

Interim Rankings

Patients: joint 26

Caregivers: 9

Health professionals: 2

T

What is the role for new methods for monitoring metabolic control (like non-invasive continuous glucose and lactate measurements, new biomarkers) for patients with liver Glycogen Storage Disease?

T

Interim Rankings

Patients: joint 40

Caregivers: joint 24

Health professionals: joint 8

U
When should liver transplantation be considered in patients with liver Glycogen Storage Disease and what are the (dis)advantages and long-term outcomes?

U

Interim Rankings

Patients: joint 48

Caregivers: 29

Health professionals: joint 5

V

Which is the role and use of medium-chain triglycerides (MCT) in the management of different patients with liver Glycogen Storage Disease?

V

Interim Rankings

Patients: joint 54

Caregivers: joint 68

Health professionals: 1