



International Niemann Pick Disease Registry

*Patient Reported Data:
How You Can Make a Difference*

INPDR

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International Niemann Pick Disease Registry

A new concept in disease registries



Welcome to the INPDR!

Niemann Pick Diseases (NPD) are rare, progressive conditions with many unanswered questions. More information is needed to better understand how they affect people and progress over time. The International Niemann Pick Disease Registry (INPDR) – a joint initiative between patient organisations and clinicians involved in the care of people with NPD – was established to better understand NPD so that potential treatments can be developed as quickly as possible.

There are two components to the INPDR, together collecting clinical, genetic, diagnostic and outcome data: patient reported data (PRD), completed by the patient or their carer if appropriate; and clinician reported data (CRD), completed by healthcare professionals caring for NPD patients.

You can confidentially record health information in the patient-reported section of the INPDR. Only anonymous health information will be made accessible to qualified researchers who are granted permission by the Steering committee. Personal health information will not be made available to employers, government organisations, insurance companies, or educational institutions. Please refer to the [Privacy Policy](#) for more information.

We welcome all patients diagnosed with any type of NPD worldwide. Patients can participate in the registry regardless of whether or not they are involved in other clinical studies and trials.

If you are registering more than one person, you may use the same login and password to enter multiple patients. However, a separate survey must be completed for each person. This can be done after registering by clicking "Add a new patient" from your log in page.

Please feel free to share any questions or concerns with us! You can contact the INPDR team at inpdr@uhb.nhs.uk

Thank you for taking the time to complete this information and assist in our efforts to improve the lives of those living with Niemann-Pick Diseases.

[Sign up](#)

[Log in](#)

The registry is owned by the International Niemann-Pick Disease Alliance (INPDA), an alliance of non-profit NPD patient support organisations. It is managed by an international consortium of professionals and is hosted at the University Hospital's Birmingham NHS Foundation Trust (UHB) in the UK. The project to develop the INPDR received funding from the European Union, in the framework of the Health Programme.

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enrol.inpdr.org

INPDR - International Niemann-Pick rare disease registry

INPDR Contact Us Log In

Sign up

* Email

* Password

8 characters minimum

* Password confirmation

Back

Submit

Log in

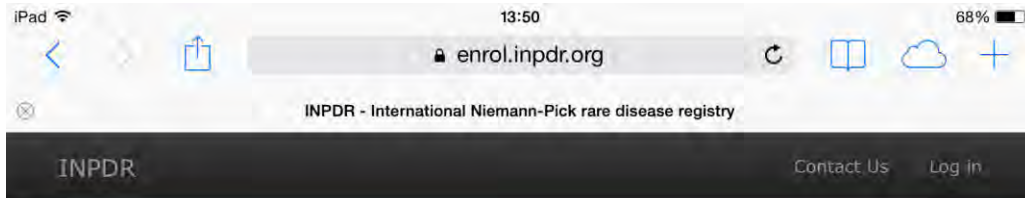
Didn't receive confirmation instructions?

Didn't receive unlock instructions?

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A message with a confirmation link has been sent to your email address. Please follow the link to activate your account.


Thank you for registering in the INPDR!


Your registration is now being processed. We will be in touch via email as soon as possible with your user name and password. You will then be able to log into the registry and complete the patient reported questionnaires.

In the meantime, if you have any questions at all you can contact us at inpdr@uhb.nhs.uk. And again, THANK YOU for your involvement.


From the INPDR team —

Confirmation instructions

 admin@inpdr.org

 Click here to download pictures. To help protect your privacy, Outlook prevented automatic download of some pictures in this message.

Sent: Fri 29/07/2016 13:47

To:  niemann-pick@zetnet.co.uk

Welcome to the International Niemann-Pick Disease Registry

You have successfully signed up to the INPDR, your username is: niemann-pick@zetnet.co.uk

Please now confirm your account email by clicking on the link below:

[Confirm my account](#)

Thank you for joining!

Welcome to the International Niemann-Pick Disease Registry Project – Patient Reported Data Registry!

Niemann Pick Diseases (NPD) are rare, progressive conditions with many unanswered questions. More information is needed to better understand how they affect people and progress over time. The International Niemann Pick Disease Registry (INPDR) – a joint initiative between patient organisations and clinicians involved in the care of people with NPD – has been established to better understand NPD so that potential treatments can be developed as quickly as possible.

There are two components to the INPDR, together collecting clinical, genetic, diagnostic and outcome data: patient reported data (PRD), completed by the patient or their carer if appropriate; and clinician reported data (CRD), completed by healthcare professionals caring for NPD patients.

You can confidentially add health information in the patient-reported section of the INPDR. Only anonymous health information will be made accessible to qualified researchers who are granted permission by the steering committee. Personal health information will not be made available to employers, government organisations, insurance companies, or educational institutions. Please refer to the Privacy Policy for more information.

We welcome all patients diagnosed with any type of NPD worldwide. Patients can participate in the registry regardless of whether or not they are involved in other clinical studies and trials.

If you are registering more than one person, you may use the same login and password to enter multiple patients. However, a separate survey must be completed for each person. This can be done after registering by clicking "Add a new patient" from your log in page.

Please feel free to share any questions or concerns with us! You can contact the INPDR team at inpdr@uhb.nhs.uk

Registration

Thank you for taking the time to complete this information and assist in our efforts to improve the lives of those living with Niemann-Pick Diseases.

*** Registrant First Name**

*** Registrant Last Name**

Address

*** Address line 1**

Address line 2

Town

*** County / province**

*** Post code / area code**

*** Country**

Relationship to Patient

Patient First Name

Patient Last Name

Patient Sex

Patient Date of Birth

Specialist

Clinician name

Hospital name

Clinician email address

Hospital phone number

Address

Address line 1

Address line 2

Town

County / province

Post code / area code

Country

Cancel

Submit

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Consent Form

Details of your involvement in the registry

Project: International Niemann Pick Disease Registry – Patient Reported Data

Before you agree to register in the INPDR, it is important that you understand what is involved and what will be done with the information that you provide. This form contains the answers to the questions that you might have. At the end of the form there is a checkbox for you to click on to confirm that you agree to participate. If you have any questions after reading this form, please contact us before continuing.

Should you have questions relating to the registry, you can contact the registry principal investigator Dr Tarekegn Hiwot (you can find his contact information below), the INPDR study team at inpdr@uhb.nhs.uk or the International Niemann Pick Disease Alliance at info@inpda.org.

What is a patient registry and why do we want to create one?

Niemann Pick Diseases (NPD) are rare, progressive conditions with many unanswered questions. More information is needed to better understand how they affect people and progress over time. The International Niemann Pick Disease Registry (INPDR) – a joint initiative between patient organisations and clinicians involved in the care of people with NPD – was established to better understand NPD to help guide researchers so that potential treatments can be developed. The registry will also help answer questions such as how NPD is distributed internationally, and will support other activities to improve patient care and establish a good standard of care worldwide.

There are two components to the INPDR, together collecting clinical, genetic, diagnostic and outcome data: patient reported data (PRD), completed by the patient or their carer if appropriate; and clinician reported data (CRD), completed by healthcare professionals caring for NPD patients.

Whose data are we collecting in this registry?

The INPDR Patient Reported Data registry is for all patients diagnosed with any type of NPD worldwide. Patients can participate in the registry regardless of whether or not they are involved in other clinical studies and trials.

Where will the data be stored?

All electronic data generated from the PRD will be held at the University of Melbourne, Australia under the direct responsibility of the Director of eResearch, Professor Richard Sinnott. Your data will be stored securely and no unauthorized users will be able to gain access to any information about you. Patient Identifiable Information that is required for enrolment will be kept in the Registry Master File which is securely held at the Queen Elizabeth Hospital Birmingham, UK, under the responsibility of Dr Tarekegn Hiwot. Patient Identifiable Information will not be held on the PRD itself.

Who will have access to the data?

The INPDR study team will have access to your data. They may be required to obtain access to your genetic diagnosis report from your medical records in order to find information necessary for your involvement in the Registry. Access to your medical records will be on your behalf, and will only occur if you have given approval for PRD staff to do so.

How often do I need to complete the questionnaires?

To ensure that a complete picture of NPD is being captured, it is recommended that you complete the follow up questionnaires every 6 months. An email will be sent to the email address you have provided to remind you that a new questionnaire is ready to be completed.

How can I update the data if something has changed?

Once a questionnaire has been completed, you are able to make changes to the questionnaire if you realise that something it needs to be corrected. We also ask that you inform us if there are any major changes in your details (e.g. change of email address) that might occur in the period between updates.

How will I be identified in the registry?

Your patient identifiable information will be securely held in the Registry Master File so that PRD staff members can contact you if it is necessary to inform you about your participation in the PRD. No personal identifiable information will be held on the electronic registry.

This information will be stored in a secure manner and your records will be assigned a unique ID. This unique ID will be used to identify your records. Authorised staff using registry will not be able to identify you personally from the information. Only the individual in charge of the Registry (Dr Tarekegn Hiwot) and persons explicitly appointed to the PRD team by Dr Hiwot will be able to link your unique ID to your personal information.

Will my data be kept confidential?

All electronic data (except for personal identifiable information) generated from the PRD will be held at the University of Melbourne, Australia under the direct responsibility of the Director of eResearch, Professor Richard Sinnott. Personal identifiable information will be stored in the Register Master File which is securely held at the Queen Elizabeth Hospital Birmingham, UK, under the responsibility of Dr Tarekegn Hiwot.

The Registry team have direct experience in working to UK and international standards (including ISO 17799, and US 21 CFR part 11), and have extensive experience of using healthcare data in the context of privacy and data protection legislature (including the Data Protection Act 1998 and EU Data Protection Directive 95/46/EC).

In order for third parties (such as researchers, clinical research companies, pharmaceutical companies etc.) to access data held in the registry, they must meet the strict requirements laid out by the Registry Management Committee, including having ethics committee approval. Approved third parties will only have access to anonymous information and will not be able to link any personal identifiable information to the data held in the registry. Your data will not be made available to employers, government organisations, insurance companies, educational institutions or to other members of your family.

How will I benefit from registering?

The registry is intended as a public service for the benefit of patients living with NPD. You will not receive any payment or any other financial benefit as a result of submitting data to the registry. Nevertheless, there are other benefits from participating, such as finding potential candidates for clinical trials, so helping speed up drug development. The data collected might also provide benefits for other patients with the disorder, for example by revealing statistics on how many people worldwide have the condition, or providing information for researchers interested in the best standards of care.

Do I have to participate in this registry and can I withdraw from it if I change my mind?

Your participation in this project is completely voluntary and you are not obliged to participate in the Registry. Additionally you are free to withdraw from the registry at any time. Should you wish to withdraw from the registry, you are not required to offer a reason why. All data generated will be securely destroyed and you will not be contacted in regards to the registry. If you wish to withdraw, you should contact inpdr@uhb.nhs.uk.

Who should I contact if I have any other questions?

If you would like any additional information about the registry, if you need to tell us about any changes in your data, or if you wish to withdraw your data from the registry, please contact the INPDR Study team at inpdr@uhb.nhs.uk or contact the registry co-ordinator:

Dr Tarekegn Hiwot

Address: Inherited Metabolic Disorders,

Department of Endocrinology

Queen Elizabeth Hospital

Queen Elizabeth Medical Centre

Birmingham

B15 2TH

Telephone: 0121-371-6982

Email: inpdr@uhb.nhs.uk

Consent

Name of Patient (if self-registering) or Parent/Guardian (if registering on behalf of a person diagnosed with NPD):

I have read and understood the patient information and informed consent document. The nature of the registry has been fully explained to me and where appropriate and possible I have discussed it with the patient whose parent or guardian I am. I have had the opportunity to ask questions, and all my questions have been answered to my satisfaction. I hereby give my consent and agree to participate in the Patient Reported Data section of the International Niemann Pick Disease Registry.

Agree

Cancel

Submit

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Signed in successfully. ✕

Welcome niemann-pick@zetnet.co.uk

Registered Patients

Registrant First Name	Registrant Last Name	Relationship to Patient	Patient First Name	Patient Last Name		
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Enrolled patients

Note. You can only create/complete a questionnaire after your enrolment information has been verified. You will be contacted via the email you provided when this has been completed. We also note that once a questionnaire has been completed, it is not possible to enter a new questionnaire until a period of 3 months have lapsed.

Patient ID	Verified	Enrolment Form	# of Completed Questionnaires		
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[Add new patient](#)

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Online Enrolment

Niemann Pick Type C

I have Niemann Pick Type C (NP-C) and would like to register myself

Behalf of Niemann Pick Type C

I would like to register on behalf of a person diagnosed with Niemann Pick Type C (NP-C)

ASMD Niemann Pick Type B

I have ASMD Niemann Pick Type B (ASMD NP-B) and would like to register myself

Behalf of ASMD Niemann Pick Type B

I would like to register on behalf of a person diagnosed with ASMD Niemann Pick Type B (ASMD NP-B)

Behalf of ASMD Niemann Pick Type A

I would like to register on behalf of a child diagnosed with ASMD Niemann Pick Type A (ASMD NP-A)

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I would like to register on behalf of a person diagnosed with NP-C

ENROLMENT INFORMATION

You are registering on behalf of a person who has been diagnosed with NP-C. If this is incorrect, please go back and select a different option. All items marked with an asterisk are compulsory fields.

Please tell us your relationship to the person you are registering

Did he/she have prolonged jaundice, liver problems, or an enlarged liver and/or spleen as a baby?

Other than the problems mentioned above, how old was he/she when other symptoms of NP-C were first observed (this may be before or after NP-C was diagnosed)? If they do not have any symptoms of NP-C, please select No symptoms.

Years

Months

No symptoms

Unknown

So we can understand how different people FIRST experience NP-C, please tell us which of the following symptoms you FIRST noticed. You can select more than one option, and provide further information if appropriate.

- Delayed milestone development
- Problems at school, not keeping up with peers
- Problems with coordination and movement
- Problems with eye movements
- Behavioural problems
- Seizures
- Psychiatric issues
- Other/further information

Please specify

How old were they when a diagnosis of NP-C was confirmed by a doctor? For example, if diagnosis was confirmed at aged 2 and a half years, select "2" Years and "6" months. If you don't know when a diagnosis was confirmed, please check the "Unknown" box.

Years

Months

Unknown

Other family members diagnosed with NP-C

Is there currently or has there been anyone else in the family diagnosed with NP-C

If yes, please tell us their relationship to the person you are registering

GENETIC DIAGNOSIS

When this person was diagnosed with NP-C, you may have received information regarding their genetic diagnosis. This tells you what mutations were found in their DNA. This may have come from a diagnostic centre or from a doctor.

Knowing the precise details of an individual's genetic mutation will add to our understanding of the condition and is likely to be important for developing treatments.

If you have a copy of the genetic diagnosis yourself (this may be a genetic report or any other document that includes details of the genetic diagnosis), please provide a copy by uploading here (please upload a PDF).

No file selected.

To remove selected file, Click Choose File then cancel

If you do not have the genetic report yourself, you may be able to obtain a copy by speaking to your specialist. After your specialist has provided this to you, please come back this form and upload the genetic report.

or

- If you are unable to contact your specialist, we will contact your specialist on your behalf using the information you provided previously regarding your specialist. If you give consent for us to do this, please tick this box.
- If a genetic test has not been performed, please tick this box

Cancel

Submit

Thank you for enrolling in the INPDR!

Your enrolment details are now being processed. We will be in touch via email as soon as possible to confirm your registration. You will then be asked to log into the registry and complete the patient reported questionnaires.

In the meantime, if you have any questions at all you can contact us at inpdr@uhb.nhs.uk. And again, THANK YOU for your involvement.

From the INPDR team —

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