

## **TREAT-NMD Global Database for SMA – feasibility enquiry**

*One of the most exciting advantages of the TREAT-NMD trial site and patient registries is the way they enable companies to conduct market analyses and feasibility Questions such as the number of patients per country fulfilling certain inclusion criteria can be answered with a targeted feasibility enquiry to our registries, resulting in valuable information for trial participation. Our registries can also be used for academic studies and trial recruitment*

This form is to be filled in by any third party requesting information from the TREAT-NMD Global Database (patient registry) for DMD.

The information given in this form is used to obtain approval from the TREAT-NMD Global Database Oversight Committee (TGDOC). Once approval is granted the request will form the basis of a contract between TREAT-NMD and the third party, which may include payment. The TGDOC commits to providing a verdict within 2 weeks. The Global Database commits to providing a full response within an agreed timeframe after approval by the TGDOC.



## Notes:

In order to evaluate your application, the TGDOC requires some information about you and a brief summary of your project plans. In many cases we may not require detail beyond what is already in the public domain. However, if a confidentiality/non-disclosure agreement is required, please request this before completing this form.

For data protection reasons, patient information will only be provided as anonymized, aggregate data ("patient numbers"). However, all patients for whom data is provided have agreed to be contacted by their national registry for recruitment purposes, and this service can be provided at the appropriate stage in your project.

For further details about the TGDOC and the principles governing the patient registries, please see the Registries Charter provided with this form. Further information is available [online](#).

**Disclaimer: Whilst every effort has been made to ensure the data collected in the TREAT-NMD Global Patient Database is accurate and up-to-date, TREAT-NMD accepts no liability for action taken on the basis of the information provided or any loss or damage thereby incurred.**

**1) About you and your enquiry**

Your company name and place of business	
How many people does your company employ?	
Contact person	
Target disease/Therapeutic area	
Is a CDA/NDA required?	Yes <input type="checkbox"/> No <input type="checkbox"/> Not at this time <input type="checkbox"/>
Detailed description of your enquiry	
Would you also like to carry out an enquiry into the Care and Trial sites registry ( <a href="#">CTSR</a> ) to identify suitable trial sites?	<input type="checkbox"/> Yes <input type="checkbox"/> No
Are you interested in using the registries as a trial recruitment tool?	<input type="checkbox"/> Yes <input type="checkbox"/> No

## 2) Countries required

Please indicate which countries you would like to receive information about:

Europe	Americas
<input type="checkbox"/> All Europe	<input type="checkbox"/> All Americas
<input type="checkbox"/> Austria	<input type="checkbox"/> Canada
<input type="checkbox"/> Belgium	<input type="checkbox"/> USA
<input type="checkbox"/> Bulgaria	
<input type="checkbox"/> Croatia	<b>Asia - Pacific</b>
<input type="checkbox"/> Cyprus	<input type="checkbox"/> All Asia-Pacific
<input type="checkbox"/> Czech Republic	<input type="checkbox"/> Australia
<input type="checkbox"/> Denmark	<input type="checkbox"/> China
<input type="checkbox"/> Finland	<input type="checkbox"/> New Zealand
<input type="checkbox"/> France	<input type="checkbox"/> India
<input type="checkbox"/> Germany	<input type="checkbox"/> Japan
<input type="checkbox"/> Hungary	
<input type="checkbox"/> Italy	<b>Rest of World</b>
<input type="checkbox"/> Lithuania	<input type="checkbox"/> All Rest of World
<input type="checkbox"/> Netherlands	<input type="checkbox"/> Algeria
<input type="checkbox"/> Poland	<input type="checkbox"/> Argentina
<input type="checkbox"/> Romania	<input type="checkbox"/> Brazil
<input type="checkbox"/> Spain	<input type="checkbox"/> Columbia
<input type="checkbox"/> Serbia	<input type="checkbox"/> Macedonia
<input type="checkbox"/> Slovakia	<input type="checkbox"/> Mexico
<input type="checkbox"/> Slovenia	<input type="checkbox"/> Russia
<input type="checkbox"/> Switzerland	
<input type="checkbox"/> Turkey	
<input type="checkbox"/> Ukraine	
<input type="checkbox"/> UK & Ireland	

## 3) Specifics of enquiry (inclusion criteria): leave boxes un-ticked if not relevant

Please indicate the inclusion criteria for this enquiry (trial):

Mandatory Items	
Genotypic inclusion criteria (please specify, e.g. precise mutation name in SMN1 gene)	
Age range	
Clinical Diagnosis	<input type="checkbox"/> Spinal Muscular Atrophy <input type="checkbox"/> Other (specify)

Ambulation	<input type="checkbox"/> Ambulant <input type="checkbox"/> Non-ambulant
Best motor function achieved	<input type="checkbox"/> Walking (age of acquisition) <input type="checkbox"/> Sitting independently (age of acquisition) <input type="checkbox"/> Never able to walk or sit independently
Wheelchair user	<input type="checkbox"/> No <input type="checkbox"/> Part-time (age) <input type="checkbox"/> Full- time (age)
Gastric / nasal feeding tube	<input type="checkbox"/> Yes <input type="checkbox"/> No
Spinal surgery for scoliosis	<input type="checkbox"/> Yes <input type="checkbox"/> No
Currently involved in clinical trial	<input type="checkbox"/> Yes, currently <input type="checkbox"/> No but previously <input type="checkbox"/> Never
<b>Highly encouraged items (available in majority of countries)</b>	
Ventilation	<input type="checkbox"/> Not ventilated <input type="checkbox"/> Part-time non-invasive ventilation <input type="checkbox"/> Full-time non-invasive ventilation <input type="checkbox"/> Part-time invasive ventilation <input type="checkbox"/> Full-time invasive ventilation
FVC	<input type="checkbox"/> Yes <input type="checkbox"/> No <input type="checkbox"/> FVC %
SMA Classification	<input type="checkbox"/> SMA 1 <input type="checkbox"/> SMA 2 <input type="checkbox"/> SMA 3
Number of SMN2 copies	<input type="checkbox"/>

*Please complete this form and return it to [Becca.leary@ncl.ac.uk](mailto:Becca.leary@ncl.ac.uk)*