

# **7<sup>th</sup> TREAT-NMD Curators' & TGDOC Meeting**

**3-4 November 2014, Leiden, Netherlands**

**Hampshire Hotel Fitland Level Leiden**



# Welcome

Dear Curators and TGDOC members,

We would like to welcome all of you in the city of Leiden for the 7<sup>th</sup> TREAT-NMD Curators and TGDOC meeting in November 2014.

Some of the previous meetings were focussed on the DMD and SMA patient registries. However, as the number of the TREAT-NMD affiliated registries, as well as the number of diseases that they cover, has grown extensively and is still expanding we hope that at this year's meeting we will have a fruitful and lively interaction among the curators from many different neuromuscular diseases.

During this year's meeting we plan to have several presentations to inform you on the progress made by various patient registries. Also, as the TREAT-NMD affiliated patient registries are being used in a growing number of enquiries and in several large projects funded by the European Union framework programmes, we will update you on those. There have also been several outstanding papers published recently and there are further publications that are currently being worked on. This year's meeting will be an opportunity to discuss these achievements and look at the way forward. There are also some new opportunities, and challenges, for the patient registries such as further integration of the registries and utilisation of the registries for post-marketing surveillance and we would like to discuss these topics during this year's meeting as well.

You can find a draft agenda for the meeting as well further details about the venue and location in this document.

We look forward to seeing you all in Leiden.

Jan Verschuuren, Chair

Hugh Dawkins, Vice-chair

## Venue

### Hampshire Hotel Fitland Level Leiden

**Address:** Bargelaan 180, 2333CW, Leiden,  
The Netherlands

**Email:** [receptieleiden@fitland.nl](mailto:receptieleiden@fitland.nl)

**Tel:** +31 71 8700 260

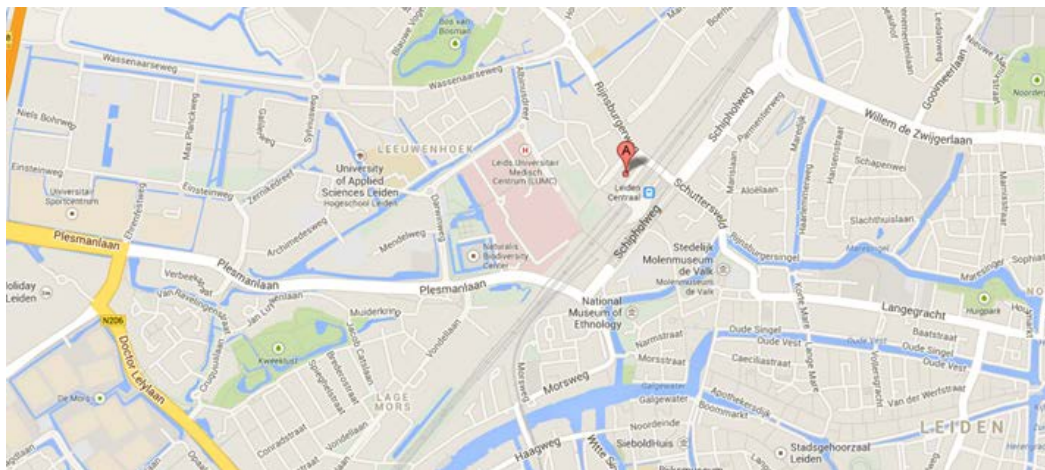
**Website:** <http://www.fitland.nl/index.php?page=1179&lang=en>



Single room= € 79,00/ Double room = € 89,00 (bed and breakfast)

**Please fill in the reservation form by 5<sup>th</sup> September to secure your room booking at the hotel.**

The hotel is conveniently located next to the train station in Leiden:



## City of Leiden

Leiden is one of the oldest cities in the Netherlands. It is located on the Rhine river and water plays an important role in the city, with its many canals. It has the oldest university of the Netherlands, which was established in 1575. Rembrandt van Rijn, the famous painter, was born in Leiden.



More information about the city can be found here: <http://portal.leiden.nl/en/homepage>

# Draft Programme

3<sup>rd</sup> - 4<sup>th</sup> November 2014

Hampshire Hotel Fitland Level Leiden, the Netherlands

## Monday 3<sup>rd</sup> November 2014 Curators' Meeting

12.00	Buffet Lunch	
<b>13.00-14.30</b>	<b>Session 1</b>	
13:00	Opening comments	Jan Verschuuren and Hugh Dawkins
13.05	TREAT-NMD Alliance and patient registries activities update	Jan Verschuuren
13.20	CTSR update	TBC
13.40	RD- Connect update	Hugh Dawkins
14.00	Registry related recent publications on DMD	Catherine Bladen
<b>14.30</b>	<b>Coffee Break</b>	
<b>15.00 -17.00</b>	<b>Session 2: Case studies of patient registries for neuromuscular disorders (~20 mins each)</b>	
	○ GNE Myopathy Registry	Oksana Pogoryelova
	○ Myotonic Dystrophy Registry	TBC
	○ Global FKRP Registry	Karen Rafferty
	○ FSHD Registry	TBC
	○ CMT Registry	TBC
	○ MTM and CNM Registry	TBC
<b>17.00-17.20</b>	<b>Coffee Break</b>	
<b>17.20 –18.20</b>	<b>Patient and ethical issues</b>	
	○ Clinical trials and expectations	Patient org. rep
<b>18.20-18.30</b>	<b>Final comments for the day - meeting adjourned</b>	Jan Verschuuren
<b>19:30–22.00</b>	<b>Dinner</b>	

## Tuesday 4<sup>th</sup> November TGDOC Meeting

<b>08.30-10.30</b>	<b>Session 3: Open Discussion</b>	Jan Verschuuren/Hugh Dawkins to lead the discussion
8.30-9.00	○ Patient feedback through the registries	
9.00-9.30	○ IT solutions and platforms for registries	
9.30-10.30	○ Post marketing surveillance	

**10.30 Coffee Break**

**10.45-12.15 Session 4: TGDOC Chair Report and Discussion**

Discussion to include:

- 6<sup>th</sup> Year report on Oversight Committee activities
- TGDOC Structure
  - New and resigned members
  - CDA and disclosures
  - Chair and chair-elect 2014-2015 - elections
- Charter - further updates and votes
- Status of the Global Registries
- Communication and information dissemination
- Date & venue of the 2015 meeting

Jan Verschuuren and  
Hugh Dawkins to lead  
the discussion

**12.15 Conclusions**

**12.30 End of meeting and adjourn for lunch**