Planning for the Future of TGDOC

Webinar FAQ Sheet

Contents

Universal Registry Platform (URP) and TREAT-NMD Enterprise

1) Could patients use the URP directly, rather than through a member registry?

In the first instance patient input into the URP will be through their TGDOC Member Registry. Limited direct access is planned as a second phase. Full direct registration by patients through the URP is currently out of scope, although we do feel that it could be advantageous to consider it, therefore our providers and developers are looking at the possibility for patients to input data directly into the URP.

2) Who has been commissioned for the build?

An information technology group based in the UK called *Wolf Group Ltd.* A formal study and procurement exercise including alternative providers was undertaken, and Wolf Group received unanimous approval from TGDOC and the TREAT-NMD Alliance Executive Committee.

3) Where will the URP server be based?

Microsoft has a portfolio of over 45 different countries where the server(s) could be based. The decision has not yet been made but is the infrastructure for the URP will allow for there to be multiple servers in Europe and the US (for example) to service the governance requirements of registries.

4) Will the URP be built to receive data inserted in other platforms?

Yes, it is our intention to build a Central Data Warehouse (CDW). The UPR will feed data directly into the CDW, from those registries that chose to use it. We will then also work with registries who have their own well-established platform/systems and do not wish to input data directly into the URP, providing them with the requirements and functionality to transfer data extracts from their platform/system, directly into the CDW.

5) Do we need to have another agreement signed to enter data into the URP ?

Yes, once the URP and CDW are built, we will be engaging with each TGDOC registry to establish how their registry data will enter the CDW and accordingly, will have appropriate Data Sharing Agreements in place

6) Is it possible to read the requirements that are now formulated?

Yes, that would be possible. A request for the information would need to be made to the TGDOC Executive Board stating who is making the request and the reason for the request. Once the TGDOC Exec Board approves the request then the information will be sent over to the requestor.

7) Will it cater for patient-reported data as well?

Yes. In the first instance patient reported data will come via the clinician inputting on behalf of the patient. In the second development phase, the patient will be able to enter limited patient details including Patient Reported Outcome Measures. Out of scope at this point, but something we want to develop quickly, is the functionality for full and independent patient entered data into the URP

8) For a country that is just about to start a national registry, is it advisable to use an independent platform first then migrate to URP, or wait for the URP?

This is a decision that only the registry can make. If it can wait approximately 10 months, the URP will be readily available to use by all registries. If the registry wished to test the URP, they can apply to become a URP pilot site, and test the URP as part of the development programme in Oct/Nov/Dec. If there is a more urgent need, the registry could consider using a cheaper solution temporarily, such as REDCap. Any registries that wish to participate in the pilot testing should contact Jo Bullivant for SMA or John McKenna for DMD (contacts listed at the end of this document)

9) If your registry is not using the URP platform, what will be the process of sharing data?

We will be developing a standardise the process of sharing data in order to ensure that the transferred data matches the data from the URP, within the Central Data Warehouse, which in turn will ensure high quality harmonised data is captured and stored. We will keep the process as simple and straight forward as we can. Data sharing agreements will be in place (see Q5 above)

Global Registry Enquiries and Datasets

10) What exactly is the role of the Enterprise regarding registry enquiries?

The role of the Enterprise is primarily around the operational delivery of enquiries, making sure we have well-functioning processes and Standard Operational Procedures (SOPs) in place. It will also manage the contracting on behalf of TGDOC with the enquiring third parties and will hold and distribute funding for enquiries as instructed by the TGDOC Chairs. The established governance processes around the enquiries will not be changed.

11) Will TREAT-NMD have access to raw data?

It is important to note that when registries choose to use the URP to host their registry, TREAT-NMD will not have access to their raw data. In this way TREAT-NMD will not have access to any Patient Identifiable Data. The data transferred to TREAT-NMD (directly via the URP or indirectly to the CDW) will only occur with the permission of the registry curator (and where appropriate to the consent

provided by the patient).

12) ***Are there any plans in the near future to update the TREAT-NMD Core Minimum datasets for DM1 and FSHD? If so when do you expect this to begin?***

We are aware that these datasets need refreshing. Typically, this has happened as part of a multi-stakeholder meeting which requires resource and funding; this is certainly something we would like to work towards for these diseases in the coming months and years.

13) ***For a Global Registry Enquiry, will only Core Member Registries have the right to vote?***

Yes, only the Core Member Registries for the disease area in question participate in Global Registry Enquiry votes.

14) ***Can Affiliated Member Registries choose to also contribute data in response to a Global Registry Enquiry?***

In principle, only Core Member Registries can contribute data as part of a Global Registry Enquiry. Affiliated Member Registries by definition do not meet the necessary criteria to fully participate in these enquiries. They may have chosen not to sign a CDA or may not be collecting the established Core Minimum Dataset items, for example.

POST WEBINAR NOTE – There may be exceptions, where Affiliated Member Registries may be encouraged to participate in surveys or informal queries coordinated by TGDOC which do not require specific Core Dataset items. These may include high-level queries on total numbers of patients, for example, or queries relating to diseases where there has not been a Core Dataset agreed as yet A CDA will be required before registries can participate. ***Please see TGDOC Enquiries and Voting SOP for further information***.

15) ***… if they DO collect the mandatory items, can Affiliated Member Registries take part in Global Registry Enquiries?***

If a registry does collect the mandatory Core Dataset items, has a CDA in place and meets the other essential criteria to take part in Global Registry Enquiries they would meet the requirements to become a TGDOC Core Member Registry. Affiliated Member status is primarily for registries that either choose not to, or are unable to, meet the criteria required to be core a Core Member Registry**.** **Please see 'POST WEBINAR NOTE' under Q14**

16) ***If an Affiliated Member Registry had a CDA and collected the Core Dataset, would they automatically become a Core Member Registry?***

Not automatically, the Registry Curator would need to complete the TGDOC Core Membership checklist and be approved by the TGDOC Executive Board.

17) Could an Affiliated Member Registry be involved in a TGDOC <u>Recruitment</u> Enquiry so that the members of that registry have the ability to participate?

In principle , only Core Member Registries can participate in any TGDOC Recruitment Enquiries as these are reliant on registries collecting the Core Minimum Datasets, as the data items are used to select which patients will receive trial information.

POST WEBINAR NOTE -–Similar to Q14 above, there may be exceptions where Affiliated Member Registries may be asked to participate in broader, less specific recruitment support activities, if Core Dataset items are not required. A CDA will be required to be in place, before this could commence. ***Please see TGDOC Enquiries and Voting SOP for further information.***

TGDOC Membership

18) Is the intent for LGMD registries to become Core Registry Members, or to remain the Affiliated group?

Yes, the intention is that LGMD registries will move into the Core Registry Members Group once the LGMD dataset project to develop and establish an approved LGMD Core Dataset is completed (*the TREAT-NMD Core Minimal Dataset for LGMD*). Registries that align with any established TREAT-NMD Core Dataset and meet the other Core Member Registry criteria will be eligible to join the Core group. The aim of the new TGDOC structure is to support all eligible registries to achieve Core Membership status, if desired.

19) How do we know whether we are in the Core group or not?

We will be sending out newly developed TGDOC Membership Packs to all our registries later in the summer, which will include checklists for Core and Affiliated Membership. If you can confidently check off all the essential requirements for Core Membership, then your registry will be approved as a Core Member. Those who do not meet the essential requirements for Core Membership will be Affiliated Members, will hopefully work towards becoming Core Members with support from TGDOC and Subgroup Leads.

20) A registry may try to update their data every 12 months, but some of the registry participants may not respond. Is there any requirement for a certain percentage of the data to be updated in order to remain a Core Member Registry?

We understand it is not always possible for all patient data in every registry to be updated annually, however this is encouraged as best practice and as such would expect the vast majority of registries to complete this at least annually. If a Core Member registry was not able to achieve this for a small percentage of patients in their registry, and could demonstrate 'good reason' why this was the case, then this would be reviewed by the TGDOC Executive Board on a case by case basis, with a view to allowing the registry to remain a Core Member, where appropriate.

It is possible we may need to implement a minimum percentage of updated data in future, for example if this is required by regulators to qualify the TREAT-NMD URP and datasets.

Please note: We do ask that only data that has been updated in the last 12 months should be included in TGDOC Global Registry Enquiries.

21) For registries holding more than one disease will it be ok to have more than one TGDOC Membership?

Registries collecting data on more than one NMD may have both Core and Affiliated Memberships for different disease areas. For example, a registry that collects data on DMD, LGMD and GNE Myopathy patients could have the following TGDOC Memberships:

- The registry collects all the mandatory items in the Core Minimum Dataset for DMD, and has completed the checklist for Core Membership, so they would be **a Core Member Registry for DMD**.
- The registry collects data on LGMD patients, but as the Core Minimum Dataset for LGMD is not yet established, they can only currently apply for **Affiliated Membership for LGMD**. As soon as the LGMD Dataset is active and the registry is collecting the mandatory data items, they can then apply for **Core Membership status for LGMD.**
- The registry collects data on GNE Myopathy patients, but as no Core Minimum Dataset exists for this disease, they are only eligible for **Affiliated Membership for GNE Myopathy**. TGDOC are developing an 'Ultra-rare' disease subgroup for registries collecting data on diseases where no TREAT-NMD Dataset exists, with the ambition to develop additional consensus-built Core Datasets in future as needed.

22) If you take the 'FAIRification' process seriously, many registries might have problems to comply with all the requirements. How will you assess the FAIR principles?

We aim to adopt, apply and extend the FAIR principles out to all registries, although we recognise this is a challenge. We are creating a Data Management and Data Quality Working Group which will help us fully incorporate these principles and create meaningful solutions that registries can apply to their own systems and processes. We will be reasonable with our expectations as to what is required/possible from registries.

23) Where can we find out about these FAIR principles if we aren't familiar with them?

Please see here for details on the FAIR Principles. Further detailed information will also be included in the TGDOC Membership Pack.

24) Patient registries may not comply with the mandatory items; do we have said criteria already in place?

Any registry wishing to apply for TGDOC Core Membership status but is not fully aligned with the criteria above should contact the TGDOC Executive Board to discuss your specific situation. Registries that align closely, but not fully with the criteria above *may* be accepted into the Core

Member Registries group at the discretion of the TGDOC Executive Board.

TGDOC Disease Subgroups

25) Will Affiliated Member Registries also be part of subgroups and be allowed to appoint Subgroup Leads?

Affiliated Member Registries should certainly be involved in the subgroups for any disease(s) they collect. The process for appointing Subgroup Leads is still under discussion but will be confirmed in the TGDOC Subgroup Terms of Reference document currently being developed.

26) Is there only one Patient Representative per subgroup?

This is one of the discussions we have been having while developing the Terms of Reference documents for Subgroup Leads and Patient Representatives. We do recognise that we need a diversity of voices and feel that it beneficial to have two Patient Representatives per subgroup and this is likely to be the approach we will take, but we are currently awaiting feedback on this issue.

27) Will the Patient Representative for the disease subgroup also be a voting member for enquires?

Yes, this is one of the most important responsibilities of the Patient Representatives. Submitting a vote is their way to represent the patient voice and to communicate their agreement or not with the Scope of Work.

28) When do you plan on holding the TGDOC subgroup meetings?

We are aiming to have a minimum of two meetings per year for each subgroup when possible, one will always be part of the TGDOC Annual Curators' Meeting held in Winter, and the other should ideally be held in Spring/Summer. The individual Subgroup Leads will be responsible for initiating the Spring/Summer meetings and will be supported by the TGDOC and the TREAT-NMD Secretariat.

Charter Overview

29) ***The Charter refers to 'TGDOC membership and Voting SOP' , when will this be circulated for review?***

This document is still being updated along with some other supporting documents mentioned in the Charter. We aim to circulate these by the end of July.

POST MEETING NOTE: Following further discussion it has been decided to replace the 'TGDOC Membership and Voting SOP' with two new documents, the 'TGDOC Voting & Enquiry SOP' and the 'TGDOC Membership SOP'. These will be circulated for review as soon as possible for review.

30) ***Considering larger countries, would it be possible to have multiple Charters and CDA's considered? Either related to each disease or to each Research Centre or only one for each country?***

This is not something we are currently considering but will bear in mind for future discussions and developments

31) ***Do you know when the CDA will be updated to include the new 'Enterprise' instead of Newcastle University?***

An agreement has been made with Newcastle University so any existing CDAs will still be valid until the end of this calendar year. After that, all CDAs will need to be directly with TREAT-NMD Services Ltd. (the 'Enterprise') from this date. Currently, any new CDAs we are issuing with registries are with the Enterprise and we will be planning to issue replacement CDAs with existing registries (via the Enterprise) in the coming months.

General Q&A

32) ***How relevant will Patient Reported registries be in the future for TREAT-NMD?***

Patient reported registries are very relevant and will always be important to TGDOC and TREAT-NMD. Indeed, we would like to see more of these types of registries becoming members of TGDOC.

33) ***How is TGDOC approaching outreach globally to identify and recruit additional members? Is there a strategy or system we can support or participate in expanding and including others who aren't already aware of TGDOC?***

At the moment we try to reach out virtually through the TREAT-NMD website, newsletter and social media, through presentations and outreach meetings at Conferences and NMD events and subgroup

meetings at disease-specific events. Where we have previously taken an opportunistic approach to promotion, the overarching TREAT-NMD Global Alliance are currently developing a formal five-year Strategy Plan which should be ready by the end of this calendar year.

Making people aware of TREAT-NMD via networking, marketing and promoting the good work that TREAT-NMD does is of utmost importance, and this includes the good work that TGDOC and the participating global registries do. We would encourage all registry curators to assist with this. If you are aware of any colleagues planning on setting up a new registry, please direct them to the TREAT-NMD website and to TGDOC, who can provide support and guidance to aligning with TREAT-NMD and becoming members

34) Where can we access the registry review survey?

You should have received a link in an email from Hannah Murray earlier this year, please let Helen know if not. We are currently reworking the Registries Review Survey to make it more user-friendly to complete and to allow us to make better use of the data provided, so future iterations of the survey may be in a different format.

Contacts

Please get in touch if you have any further questions or require any more information on any TGDOC matters:

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