INPDR Data release for therapy comparison studies

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0.1	16 March 2021	Conan Donnelly	
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Principles for data release for therapy comparison studies

The following document detailsthe principles that the INPDR Board will adhere to with regard to the provision of data and statistical support in research that involves the comparison of individual therapies or therapy combinations.

The priniciples relate to use of core registry data only and do not relate to the processing of trial datasets or other datasets acquired by the INPDR. Guidance for such studies can be found in the INPDR Data Migration Policy.

1. The INPDR is committed to ensuring that the data generated through its registry should be put to maximum use to deliver patient and eventual public benefit. We will therefore share data for research and other analytical activities that aim to improve outcomes in Niemann-Pick diseases, whilst safeguarding the privacy of patients.
2. The INPDR provides a whole patient view and we wish to encourage the use of the data in ways to maintain this perspective.
3. The registry shall not restrict data access or analysis to individual drugs in such a way as to render the data to that of a product registry. This principle shall be applied to all stakeholders whether they have a commercial interest in a specific therapy or not.
4. This approach shall be underpinned by the following measures intended to mitigate challenges related to relationship management, scientific independence, integrity and public confidence in research output using registry data:
    a. A signed agreement will be required to ensure that users of INPDR data fulfil the obligations stipulated prior to sharing and, that data is used only for the specific purposes for which it was intended.
    b. Users must adhere to the analysis plan as set out in the signed agreement. Analyses undertraken outwith shall not be released without the authorisation of the INPDR Board.
    c. In studies involving comparative effectiveness of therapies or therapy combinations, the INPDR shall specifically consider the limitations of observational study design in producing high quality evidence, particulary in regard to the quality and availability of co-variate data and statistical methods to manage bias. This may lead to recommendations including, but not limited to:
        i. Suggested amendments to the study design
        ii. Conditions or restrictions to the kind of data that can be made available
        iii. The need for INPDR to undertake a data quality assessment prior to sharing or to provide clear statements regarding limitations in methodology
        iv. Rejection of the request to undertake such analysis. For declined requests, the grounds for rejection will be explained and modifications that might enable approval of the research protocol offered. Options for re-submission will be specified.
        v. The INPDR Board & Scientific Advisory Committee may also choose to seek independent statistical or scientific review in the consideration of data access for such studies.

d. Whilst the INPDR may approve a research study following application of the measures set out above, this approval does not represent endorsement of a research study or its findings. The INPDR is **NOT** responsible for the quality, integrity of findings of the research of others.
e. Such comparison studies shall require an agreement that the researcher provide any related therapy owner access to draft publications 30 days in advance of submission for publication. The purpose of such sharing of publications is not to seek changes or prevent publication, rather, to provide early access for communication purposes.
f. The INPDR will act as the central point of contact for each party in this regard.
g. The INPDR will **not** act as an arbiter on quality and integrity of scientific research output. Rather, the registry shall expect that the peer review process and scientific debate shall provide the controls to ensure high standards of scientific quality are upheld for all research including INPDR led research.
h. The INPDR expects that all study results will be published, to maximise the long-term value of the data. In any reported or published findings the INPDR must be appropriately acknowledged. If for any reason the results cannot be disclosed in a peer-reviewed publication or in a manner consistent with the original plan, the INPDR will request a brief summary the, to include the reason for non-completion and the outcome of the analysis/activity performed. This should be disclosed in an open access journal or on a publishing platform, with a link made available on the INPDR website.