Improving specificity in gene therapy through nanobody coupling of AAV vectors.

A Data Management Plan created using DMPonline.be

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Template: KU Leuven BOF-IOF

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ID: 198539

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Project abstract:

Gene therapy (GT) using recombinant adeno-associated virus (rAAV) is a highly promising therapeutic modality that is seeing rapid expansion. However, it still suffers from several drawbacks inherent to an emerging therapeutic modality. For instance, the low transduction efficiency and broad tropism of rAAV leads to high dose requirements, which in turn leads to high manufacturing costs and possible toxicity. Several attempts at modulating or improving rAAV tropism have been performed, but these are either tailored to a specific disease target (necessitating time- and resource-intensive product optimization for each disease target) or suffer from poor production yield and incompatibility with cGMP production requirements. In this project, we will develop an innovative rAAV conjugation strategy to manufacture rAAV particles that can be retargeted to specific tissues or cell types and detargeted from non-target tissues. The envisioned technology is modular, and uses products and technologies that are already being used in FDA/EMA-approved drug manufacturing and drug products. The conjugated rAAVs are highly valuable from a scientific, clinical and economical perspective and thus highly valorizable.

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Research Data Summary

List and describe all datasets or research materials that you plan to generate/collect or reuse during your research project. For each dataset or data type (observational, experimental etc.), provide a short name & description (sufficient for yourself to know what data it is about), indicate whether the data are newly generated/collected or reused, digital or physical, also indicate the type of the data (the kind of content), its technical format (file extension), and an estimate of the upper limit of the volume of the data.

Dataset name / ID	Description	New or reuse	Digital or Physical data	Data Type	File format	Data volume	Physical volume
		Indicate: ***N****(ew data) or* ***E****(xisting data)*	Indicate: **D**(igital) or **P**(hysical)	Indicate: **A**udiovisual **I**mages **S**ound **N**umerical **T**extual **M**odel **SO**ftware Other (specify)		Indicate: <1GB <100GB <1TB <5TB >5TB NA	
vectors (DNA)	plasmids encoding cap sequences	N	P	DNA	/	/	/
vectors (AAV)	AAV viral vectors	N	P	viral vector	/	/	/
analytics	Vector analytical data associated with each AAV prep	N	D	I/N/T	.ddpcr, .tif, .xls, .ppt	<1GB	ELN, J drive
FACS data	Flow cytometry data regarding in vitro experimentation	N	D	N/T	.fcs	<1GB	ELN, J drive
BLI data	mouse BLI data	N	D	I/N/T		<100GB	ELN, J drive, L drive
mouse tissue	processed mouse tissue samples (slices, frozen, lysate)	N	P	processed tissue	/	/	/

If you reuse existing data, please specify the source, preferably by using a persistent identifier (e.g. DOI, Handle, URL etc.) per dataset or data type:

No data will be re-used

Are there any ethical issues concerning the creation and/or use of the data (e.g. experiments on humans or animals, dual use)? If so, refer to specific datasets or data types when appropriate and provide the relevant ethical approval number.

- Yes, animal data (Provide ECD reference number below)

ECD 037/2023

Will you process personal data? If so, please refer to specific datasets or data types when appropriate and provide the KU Leuven or UZ Leuven privacy register number (G or S number).

- No

Does your work have potential for commercial valorization (e.g. tech transfer, for example spin-offs, commercial exploitation, …)? If so, please comment per dataset or data type where appropriate.

- Yes

Spin-off in advanced stadium of creation. All generated data will be part of a Tech Transfer agreement and a Research Collaboration between spin-off and the lab will be set up. Data transfer to Spin-off coordinated with lab, LRD and spin-off mgmt. C3 funding will be closed down upon Spin-off creation.
PCT application will be submitted before 28/04/2023 (end of priority year).

Do existing 3rd party agreements restrict exploitation or dissemination of the data you (re)use (e.g. Material or Data transfer agreements, Research collaboration agreements)? If so, please explain in the comment section to what data they relate and what restrictions are in place.

- Yes

A Research Collaboration is being drafted between a (to be incorporated) spin-off CO and the lab. After that, all data on the platform will transfer to the spin-off, the C3 grant will cease and all further data will be generated under the Research Collaboration, meaning that data ownership will lie at the spin-off.
Internal research rights of course hold, but that work will never overlap with the C3 grant.
This related to all data.

Are there any other legal issues, such as intellectual property rights and ownership, to be managed related to the data you (re)use? If so, please explain in the comment section to what data they relate and which restrictions will be asserted.

- Yes

See above regarding Spin-off creation.
IP will licensed and later transferred to the spin-off.

Documentation and Metadata

Clearly describe what approach will be followed to capture the accompanying information necessary to keep data understandable and usable, for yourself and others, now and in the future (e.g. in terms of documentation levels and types required, procedures used, Electronic Lab Notebooks, README.txt files, codebook.tsv etc. where this information is recorded).

The lab works with an ELN (eLabNext) wherein all data is collected. Raw data files, as well as e.g. ppt files with graphical summaries, are stored on a J drive that mirrors the ELN structure. The promoters of the grant make sure that all data is clearly described before signing off the experiments in the notebook.
SOPs that are used in the lab, are managed by the Analytics Lead, and version control is in place.
No paper notebooks are used.

Will a metadata standard be used to make it easier to find and reuse the data?
If so, please specify which metadata standard will be used.

If not, please specify which metadata will be created to make the data easier to find and reuse.

- No

All metadata is described in the ELN.

Data Storage & Back-up during the Research Project

Where will the data be stored?

- Shared network drive (J-drive)
- Large Volume Storage
- Other (specify below)

Most data is stored on the J drive and the ELN. Large files go on the Large Volume Storage drive (e.g. histological images).

How will the data be backed up?

- Standard back-up provided by KU Leuven ICTS for my storage solution
- Other (specify below)

ELN (eLabNext) is backed-up on European servers.

Is there currently sufficient storage & backup capacity during the project?

If no or insufficient storage or backup capacities are available, explain how this will be taken care of.

- Yes

How will you ensure that the data are securely stored and not accessed or modified by unauthorized persons?

ELN access is managed well, and backed up on European servers.
Access to J drive is subject to KULeuven restrictions, and only relevant colleagues can access the folders.

What are the expected costs for data storage and backup during the research project? How will these costs be covered?

Costs are minimal. Costs of the ELN are covered by internal lab funding.

Data Preservation after the end of the Research Project

Which data will be retained for 10 years (or longer, in agreement with other retention policies that are applicable) after the end of the project?

In case some data cannot be preserved, clearly state the reasons for this (e.g. legal or contractual restrictions, storage/budget issues, institutional policies...).

- All data will be preserved for 10 years according to KU Leuven RDM policy

Where will these data be archived (stored and curated for the long-term)?

- Large Volume Storage (longterm for large volumes)

- Shared network drive (J-drive)

What are the expected costs for data preservation during the expected retention period? How will these costs be covered?

Costs are minimal and will be covered by internal lab funds.

Data Sharing and Reuse

Will the data (or part of the data) be made available for reuse after/during the project?
Please explain per dataset or data type which data will be made available.

- No (closed access)

As the data will transfer to a spin-off, the data will not be shared outside of the lab.

If access is restricted, please specify who will be able to access the data and under what conditions.

Only people engaged in the Research Collaboration with the spin-off (from both KULeuven and the Spin-off side) will have access to the data.

Are there any factors that restrict or prevent the sharing of (some of) the data (e.g. as defined in an agreement with a 3rd party, legal restrictions)?

Please explain per dataset or data type where appropriate.

- Yes, intellectual property rights
- Yes, other

No data that is subject to patenting is shared.
All data will transfer to a spin-off under a Tech Transfer Agreement, as negotiated between LRD and the spin-off.

Where will the data be made available?

If already known, please provide a repository per dataset or data type.

- Other (specify below)

Data sharing is managed through a secure data vault (digify.com).
IP-sensitive data is deposited in an iDepot where and when necessary.

When will the data be made available?

- Upon publication of research results

Upon obtaining IP, and in agreement with the spin-off, the data will be made available to the public in the form of posters/presentation/papers.

Which data usage licenses are you going to provide?

If none, please explain why.

- Other (specify below)

Data will be shared with the spin-off through a bespoke Tech Transfer agreement.

Do you intend to add a persistent identifier (PID) to your dataset(s), e.g. a DOI or accession number? If already available, please provide it here.

- No

What are the expected costs for data sharing? How will these costs be covered?

Costs are expected to be negligible.

Responsibilities

Who will manage data documentation and metadata during the research project?

The promoters of the grant will manage data documentation:
prof. dr. Els Henckaerts
dr. Benjamien Moeyaert
prof. dr. Maarten Dewilde

Individual lab members are aware of their expected contribution to proper RDM.

Who will manage data storage and backup during the research project?

The promoters of the grant will manage data documentation:
prof. dr. Els Henckaerts
dr. Benjamien Moeyaert
prof. dr. Maarten Dewilde

Individual lab members are aware of their expected contribution to proper RDM.

Who will manage data preservation and sharing?

The promoters of the grant will manage data preservation and sharing:
prof. dr. Els Henckaerts
dr. Benjamien Moeyaert
prof. dr. Maarten Dewilde

For data sharing, LRD is setting up tech transfer agreements and guiding us in which data to share and which not.

Who will update and implement this DMP?

The promoters of the grant will update and implement this DMP:
prof. dr. Els Henckaerts
dr. Benjamien Moeyaert
prof. dr. Maarten Dewilde