UshTher
Innovative Gene Therapy for USHIB Retinitis Pigmentosa

Detailed Dissemination Plan

Deliverable 7.1

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1. Introduction

This Deliverable is the result of task 7.1. The Dissemination Plan has the aim to present and discuss the set of actions and activities planned and developed to ensure high visibility, accessibility and promotion of the UshTher project and its results.

This deliverable has been developed with two different purposes:

- to map out the dissemination strategy and outreach efforts during the project period;
- to define the audience, the messages to be disseminated, the tools and the timeline.

The DP is designed to identify 4 fundamental points:

- **What to disseminate?** In section (2) the MESSAGE to disseminate is described.
- **Who to disseminate to?** In section (3) the AUDIENCE to whom the dissemination is aimed is described.
- **When to disseminate?** In Section (4) a CALENDAR and PLAN of dissemination is described.
- **How to disseminate?** In section (5) the TOOLS and MECHANISMS of dissemination are described

An additional section (6) describes the list of the finalized, ongoing and planned dissemination activities.

2. The message: what to disseminate?

The main purpose of the UshTher DP is to ensure that the project research and practical outcomes are widely disseminated to the appropriate target audiences, at appropriate times along the project lifecycle, and particularly at key milestones, via appropriate methods, and that those who can contribute to the development and exploitation of the UshTher project outcomes can be identified and encouraged to interact with the project on a regular and systematic basis.

2.1. General information about the project

The overall objective of UshTher is to build on this data and develop a phase I/II, first-in-human, clinical trial of gene therapy for USHIB retinitis pigmentosa based on dual AAV. UshTher is highly innovative as it would be the first time that dual AAV vectors are tested in humans, or that any combination of two independent gene therapy vectors are delivered in vivo to patients. This could pave the way for using a similar approach for other devastating diseases due to large genes deficiency. In order to make UshTher

results useful, they should be of interest and easily accessible to all possible end users. The audience needs to be informed about the project, its progress, its results, its outputs and its legacy.

2.2. Research findings

UshTher produces databases, information and new knowledge that will outlast the project itself. The project will provide a first-in-human demonstration that gene therapy for USHIB retinitis pigmentosa is both safe and effective using a novel strategy based on dual AAV vectors. This would be a significant advancement as there is currently no approved treatment for any retinitis pigmentosa. The idea is based on solid pre-clinical data in animal models that demonstrates the approach is feasible and safe.

The success of the UshTher clinical trial will open new avenues for treatment of other retinal diseases, like Stargardt, USH2A or retinitis pigmentosa due to EYS mutations which are common inherited blinding conditions which require large gene delivery to the retina. Importantly, the demonstration provided by UshTher that dual AAV vectors are safe and effective in humans will greatly reduce the limitation imposed by the limited AAV packaging capacity and prompt testing of this gene therapy platform for other genetic or non-genetic diseases that benefit from large gene transfer, like Duchenne muscular dystrophy, hemophilia A or cystic fibrosis.

2.3. Findings relevant to economic and commerce

The overall UshTher objective is to define the safety and efficacy of a single subretinal administration of dual AAV vectors as therapy of USHIB retinitis pigmentosa. This is part of a larger and long-standing effort to make the therapy available to USHIB patients worldwide. Developing a cure for USHI retinitis pigmentosa will lead to significant societal and economic benefits.

With an overall prevalence of 1 in 6000 (Kimberling et al., 2010), the autosomal recessive USH syndrome is the most common combination of genetic deafness and blindness (Gregory-Evans et al., 2013). USHI, which accounts for around 40 % of all Usher cases (Rosenberg et al., 1997), is the most severe USH with the retinitis pigmentosa onset during childhood (Gregory-Evans et al., 2013). USHIB accounts for 35-50% of USHI (Ouyang et al., 2005), with an estimated prevalence of 1 in 30-42000 individuals. Considering a EU population of approximately 500 million, there are 12,500 individuals within the EU currently suffering from USHIB. The availability of a cure, especially if delivered before the retinal degeneration is complete, would therefore relieve a significant number of individuals from severe hypovision/blindness.

The October 2010 report from the Italian branch of the International Agency for Blindness Prevention (IAPB) has established that the yearly individual cost of a blind person is close to 21,000 euro (Gruppo di Ricerche Industriali e Finanziarie GRIF “Fabio Gobbo” and Carli", 2010). Thus, the annual costs of USHIB retinitis pigmentosa for the EU is up to 294M euro. The availability of a one shot treatment for USHIB retinitis pigmentosa would significantly decrease these costs. In addition, the market size for a gene therapy product for USHIB retinitis pigmentosa has been already calculated by Oxford Biomedica to be $40M per year (www.oxfordbiomedica.co.uk).

3. The audience: who to disseminate to?

Stakeholder engagement is the key to the success of any dissemination initiative, and stakeholder identification is the first and foremost important task in effective stakeholder engagement. UshTher dissemination activities involve four target stakeholder groups:

- **research community** including both biologists and physicians
- **clinical end-users and health care practitioners** (ophthalmologists)
- **private sectors:** industries and SMEs in healthcare and pharmaceutical sectors interested in the uptake of the new knowledge and technology produced
- **public administrations**, health authorities and policy makers
- **society at large** composed by patient, citizens and professionals.

4. The timeline: when to disseminate?

The objectives of the dissemination activities are mainly deployed in stages during the project lifetime. Dissemination and communication actions will be organized as follows:

1. **Starting phase (M1-M18): Raising awareness** of project’s activities, outputs and benefits through diverse channels to audiences. During this initial phase, the Consortium will elaborate the project identity and produce promotional material. This phase is still in progress.
2. **Steady phase (M18-M48): Promoting a deeper understanding of new knowledge and results.** During this central phase, as research activities will progress further, UshTher Consortium will

produce reports, datasets, publications and present the results to the scientific community. This will be the core base material for dissemination.

3. **Capitalizing phase (M48-M60): Engaging with target groups** to encourage their willingness to make use of project results.

5. The tools and mechanism: how to disseminate?

5.1. Internal Communication

For internal communication among project members, we will promote the use of IT tools. Direct contact will be maintained by **mail** to facilitate the communication among beneficiaries. We created a **dedicated section of the website** restricted to project members, to share project documents. In addition, there will be **annual meetings** between members of the project and midterm meetings between PIs and WP leaders. The Coordinator will hold talks by **phone or Skype conferences** with partners as often as needed to guarantee optimal information flow and implementation.

5.2. External Communication

5.2.1. Website

The infrastructure for the UshTher Project website (www.UshTher.eu) is provided by FTELE.IGM, who is in charge of its design and management. Since its release (see Deliverable 6.2), it is continually evolving and increasingly serving as the key information source for both project partners and external visitors. The project website will continuously evolve and develop as the project itself matures. The UshTher webpage provides the necessary function to act as communication and dissemination tool, internal networking platform and information resource. The Project Management Team will continue to manage, extend and improve its usability and functions as needed throughout the project duration.

The home page provides a short introduction to the project and includes the following sections:

- **About**: this page describes the main goals and activities of the UshTher project and the Consortium
- **News**: general news about the project
- **Events**: events organized by the UshTher partners and project meetings
- **Products**: this area will include all downloadable material that will be produced and developed during the UshTher project: deliverables, public reports and promotional material

- **Publications**: list of scientific publications, with links to the original publications or ZENODO entry
- **Internal Use**: The role of the restricted area for partners is to have a secure and private place to share official documents and information among partners.

The project website will continuously evolve and develop as the project itself matures. The UshTher webpage provides the necessary function to act as dissemination tool, internal networking platform and working and discussion space and information resource. The coordinator will continue to manage, extend and improve its usability and functions as needed throughout the project duration.

5.2.2. Twitter account

Social networking is part of the UshTher Communication strategy (see Deliverable 8.3). At the moment of writing this deliverable (January 2019), we are about to launch the UshTher Twitter account that will be used to tweet UshTher relevant information and news interesting for the scientific, private and general community. Additional social media platforms, such as LinkedIn and Facebook, will be considered as the project progresses, if deemed appropriate.

5.2.3. Open Access

The main objective of this task is to permit the free download of UshTher results and outputs.

In order to have a repository of scientific papers, data and metadata produced during UshTher, we will create a “UshTher Community” in ZENODO, the OpenAIRE ‘orphan repository’. Additional details on Open Access and Data Management are described in the Deliverable 6.13 “Data Management Plan”.

6. Communication activities

6.1. List of dissemination and communication activities – planned

Activity	Event	Date/Location	Weblink
Poster	European Society of Gene and Cell Therapy	Edinburgh, 2020	https://www.esgct.eu/Congress/Edinburgh-2020.aspx
Presentation	European Society of Gene and Cell Therapy	2021	https://www.esgct.eu/Congress/Upcoming-Congresses.aspx
Presentation	Association for Research in Vision and Ophthalmology (ARVO)	28 April – 2 May 2019, Vancouver	https://www.arvo.org/annual-meeting/
Summer School			
Workshop			
Special issue			

6.2. List of dissemination and communication activities – achieved

Activity	Event	Date/Location	Weblink
Publication	Clinical trial. gov	22-25 October 2019, Barcelona	https://clinicaltrials.gov/ct2/results?recrs=ab&cond=NCT03814499&term=&cntry=&state=&city=&dist=
Presentation			
Summer School			
Workshop			
Special issue			
Special issue			
Special issue			