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SIG SPECIAL INTEREST GROUP | REPORT

Hospital pharmacist's preparedness for in-vivo gene therapy medicinal products

June 2023

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The work of this Special Interest Group (SIG) was financially supported by Pfizer.

Foreword by the President

Gene therapy medicinal products (GTMP) offer a novel approach to treating rare and sometimes life-threatening genetic diseases and may require new responsibilities for pharmacy practice. These approaches include replacing a non-functional gene with a functioning healthy gene, inactivating a disease-causing gene, or introducing a new or modified gene into the body.

The potential and research on gene therapies are considerably increasing, especially for cancer patients and patients suffering from rare diseases. The boundaries of research have shifted significantly over the past years and hospital pharmacists are more and more involved in this kind of research. As medicines experts, hospital pharmacists should be part of the teams working with and handling in-vivo gene therapy medicinal products. Therefore, EAHP decided to put together a Special Interest Group to evaluate the hospital pharmacist's preparedness for in-vivo gene GTMP.

On behalf of EAHP, I would like to thank all SIG members for their valuable contributions and their engagement with this report which helped better understand the hospital pharmacy preparedness for handling in-vivo GTMP in Europe. Also, I would like to thank the SIG members for agreeing to continue its work to update the 2007 "EAHP Guideline on the Pharmacy Handling of Gene Medicines". The updated guideline is planned to be published by Fall 2023.

My thanks also extend towards the chief pharmacists across Europe and EAHP's member associations that contributed to the survey activity of this SIG on Spring 2022.

Andras Süle
EAHP President

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Executive Summary

This report presents the findings of the Special Interest Group on Hospital Pharmacist's Preparedness for In-vivo Gene therapy medicinal products (GTMP). This SIG was financially supported by Pfizer and set up by the European Association of Hospital Pharmacists (EAHP). The SIG carried out desk research and a survey to gather information on the hospital pharmacy preparedness for gene therapy medicines throughout Europe and on preparing and optimising multidisciplinary care teams for the integration of gene therapy medicines into the treatment tools for patients with rare diseases. The report includes a proposal for future training activities on GTMP for hospital pharmacists and identifies the need for an update of the former version of the EAHP Guidance for Handling GTMP published in 2007.

The number of gene therapy treatments available both in clinical trials and as licensed medicines are increasing, especially for cancer patients and patients affected by rare diseases. As of Quarter 4 2022, there were over 500 investigational gene therapy medicines in clinical development globally[1], mainly for rare genetic disorders and cancer. There are over 20 approved GTMP on the global market today.

The increase of GTMP clinical trials and marketed drugs is an important consideration for hospital pharmacy services. GTMP are different from traditional biopharmaceuticals and small molecule medicines. Given the complex nature of these medicines, the adoption of GTMP will require new knowledge, skills and competencies for pharmacy professionals at different levels: clinical governance, operational delivery and clinical pharmaceutical care. GTMP are associated with a complex supply chains as well as demanding storage and traceability requirements. Specialist aseptic preparation requires dedicated and appropriate aseptic isolators, biological safety cabinets or capacity to use them on a sessional basis.

The SIG survey found a wide range of hospital pharmacy preparedness for GTMP across Europe. For instance, hospitals in some countries to date do not use GTMP in clinical practice or even expect to use GTMP in the near future. Other countries like the United Kingdom are more advanced with national guidance on handling and administration of these medicines and many hospital pharmacists already trained. The survey highlighted that there is an educational need for European hospital pharmacists on all aspects of handling GTMP. We advocate for expanding training opportunities within the EAHP for the pharmacy workforce including webinars, training sessions in the annual conference and a masterclass. These educational programs should provide knowledge to enhance the capability of pharmacists to lead institutional preparedness for safe and effective delivery of GTMP when these become available in their hospitals.

[1] ASGCT Citeline Q4 2022 report

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In the absence of guidance on GTMP in hospital pharmacies in most European countries, the SIG group has agreed to update the 2007 EAHP Guidance on the Pharmacy Handling of Gene Medicines in the hospital pharmacy setting according to the current evidence and their expertise, the updated guideline will be published in Fall 2023.

Background

GTMP offer a novel approach to treating rare and sometimes life-threatening genetic diseases and may require new responsibilities for pharmacy practice. Gene therapy is defined as the introduction, change or removal of genetic material or modification of gene expression to alter the biological function of an individual's genetic code with the objective of achieving a therapeutic benefit. Mechanisms of GTMP can include gene replacement therapy, in which an functioning gene is introduced to replace a non-functioning gene; gene addition therapy for complex cancerous and infectious diseases, in which a new gene is introduced into the body to help fight disease; gene inhibition/silencing therapy or "knockdown" to inactivate a mutated gene that is overproducing its product by targeting ribonucleic acid (RNA); and gene editing therapy that permits targeted changes to a gene sequence.

GTMP can be delivered either in vivo or ex vivo. In ex-vivo gene therapy a patient's cells (e.g. hematopoietic stem cells) are harvested, the gene is inserted into these cells in the lab, and the modified cells are then reintroduced into the patient. In the case of in-vivo gene therapy, a vector carrying the therapeutic genetic material is administered directly into the patient. The vector can be administered by a variety of methods, including direct infusion into the blood (intravenous infusion), by infusion/injection into target organs or by other physical means of administration (hypodermic injection, aerosol, intrathecal delivery, etc.).[2]

The number of GTMP available both in clinical trials and as licensed medicines are increasing, especially for cancer patients and patients affected by rare diseases. Hospital pharmacists are involved in the use of gene therapy treatments both in clinical research as well as licensed products. Therefore, the European Association of Hospital Pharmacists (EAHP) set up a Special Interest Group (SIG) to evaluate the hospital pharmacists' preparedness for in-vivo gene therapy medicinal products. Its work is financially supported by Pfizer. The decisions and outcomes delivered by this working group remain independent of this financial support.

This SIG started its work in January 2022 and concluded its activities in February 2023. The activities of the SIG were aimed at the collection of information on preparing in-vivo gene therapy treatments and optimizing multidisciplinary

[2] Glossary | ASGCT - American Society of Gene & Cell Therapy | ASGCT - American Society of Gene & Cell Therapy Gene Therapy Basics | ASGCT - American Society of Gene & Cell Therapy |

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care teams for the integration of GTMP into the treatment pathways for patients with rare diseases. In addition, the group members compared the requirements for handling, preparation, and administration of GTMP throughout Europe and collated information on existing educational material available for hospital pharmacists.

In addition, the SIG will work on updating the 2007 EAHP "Guidance on the Pharmacy Handling of Gene Medicines" which will be published in Fall 2023.

The work of the SIG focused on hospital pharmacists' preparedness for GTMP and interlinks closely with the European Statements of Hospital Pharmacy aligned with EAHP's activities.

The list of SIG members can be found in Appendix I.

European Statements of Hospital Pharmacy

In 2014 EAHP, adopted the European Statements of Hospital Pharmacy[3] that express commonly agreed objectives which every European health system should aim for in the delivery of hospital pharmacy services. The topic of the handling of gene therapy is linked to several statements in the European Statements of Hospital Pharmacy cited verbatim, below:

Statement 1.1 "*The overarching goal of the hospital pharmacy service is to optimize patient outcomes through working collaboratively within multidisciplinary teams in order to achieve the responsible use of medicines across all settings.*"

Statement 2.2 "*Hospital pharmacists should take the lead in developing, monitoring, reviewing and improving medicine use processes and the use of medicine related technologies. Responsibility for using these processes may rest with other health care professionals and may vary according to the medicine, the medicine related technology, the health care setting and the multidisciplinary team delivering care*."

Statement 5.1 ""*The "seven rights" (the right patient, right medicine, right dose, right route, right time, right information and right documentation) should be fulfilled in all medicines-related activities in the hospital."*

Horizon Scanning

Throughout February, March and April 2022, the members of the SIG conducted a horizon scanning exercise to identify guidance materials, best practices and other documents that exist for hospital pharmacists on the topic of in-vivo gene therapies. The results of this exercise showed that very limited guidance material exists within Europe. Materials and

[3] www.statements.eahp.eu

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resources identified within the countries covered by the SIG (Belgium, Spain, France and the United Kingdom) included:

- e-Learning modules on advanced therapy medicinal products (ATMPs) for healthcare professionals including pharmacists in the UK[4]
- an overview of resources for hospital pharmacists prepared by one of the SIG members
- a pan-UK pharmacy working group on ATMPs[5]
- Advanced Therapy Treatment Centre (ATTC) resources in the UK[6]
- a protocol for the preparation of Luxturna® (Voretigene neparvovec) in Belgium and Spain
- SFPO (French Society of Oncology Pharmacy) recommendations and guidelines[7]
- a protocol for the preparation of Zolgensma® (Onasemnogene abeparvovec) in Belgium

At European level, the SIG recognised the existence of EAHP's Guidance on the Pharmacy Handling of Gene Medicines from 2007 which was co-authored by two of the SIG members.[8] The SIG acknowledged that this EAHP guidance required an update. A survey investigating the preparedness of hospital pharmacies for GTMP in the UK had been undertaken and published by one of the SIG members.[9]

Survey on the preparedness of Hospital Pharmacists for in-vivo gene therapy medicines

Survey design

The SIG conducted a Survey to analyse and assess the knowledge of the hospital pharmacy profession in Europe for GTMP[10] and obtain information on the preparedness of hospital pharmacy departments for the delivery of GTMP to patients. The survey was launched on the 6th of May 2022 and included questions related to the hospital pharmacy facilities and the pharmacists working in these facilities. Hospital pharmacists that are not yet working with GTMP were also encouraged to participate in this survey. The survey closed in June 2022.

[4] https://www.e-lfh.org.uk/programmes/advanced-therapy-medicinal-products/
[5] Advanced therapy medicinal products – SPS - Specialist Pharmacy Service – The first stop for professional medicines advice
[6] https://www.theattcnetwork.co.uk
[7] https://www.sfpo.com/wp-content/uploads/2015/05/Recommandations-MTI-V12-5-mai-2015vf-site.pdf
[8] Vulto, A.G., Stoner, N., Pharm, D.C., Cercós, A., Hoppe-Tichy, T., Nydert, P., Vermes, A., & Wolfsberger, A. (2007). European Association of Hospital Pharmacists (EAHP) Guidance on the Pharmacy Handling of Gene Medicines.
[9] Stoner, N., Black, A. (2020). Pan UK Pharmacy Working Group for ATMPs - Pharmacy Institutional Readiness for In-vivo (virus based) Gene Therapy Medicinal Products. Guidance for Chief Pharmacists. July 2020, Version 1. Available from: https://www.sps.nhs.uk/wp-content/uploads/2020/07/Pharmacy-Institutional-Readiness-for-in-vivo-virus-based-Gene-Therapy-Medicinal-Products-V1-July-2020.pdf
[10] Gene therapy is often defined as the introduction or removal of genetic material or modification of gene expression to alter the biological function of an individual's genetic code with the objective of achieving a therapeutic benefit. These approaches include replacing a non-functional gene with a functioning healthy gene, inactivating a disease-causing gene, or introducing a new or modified gene into the body.

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All information obtained was kept confidential and will not be used to identify any specific hospitals or individuals.

The questionnaire was organised into themes and included 45 questions. There was opportunity to collate additional details from hospitals providing GTPM, as well as information from hospitals not yet involved in providing these treatments.

Respondents

The Survey was available to all chief pharmacists from EAHP's member countries via EAHP's 35 national associations. In addition, the Survey was available on the EAHP website and promoted via social media from April to June 2022.

There were 216 responses to the Survey. There was a considerable difference in percentage of respondents from the United Kingdom (n=34), France (n=31) and Portugal (n=28). **These countries have a high percentage of respondents thus some results might not show a fully representative picture as some answers come from only a few of EAHP countries as seen below.**

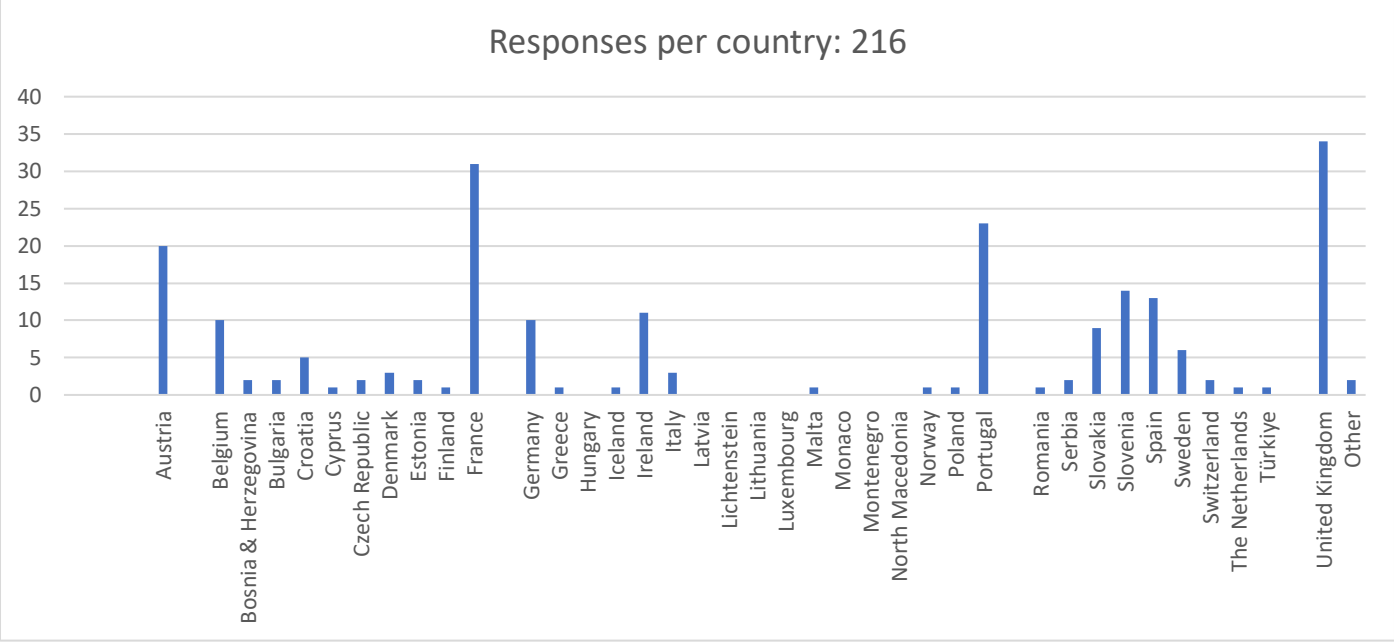

Figure 1: Overview of the country from the respondents that participated in the Survey

More than three-quarters of respondents worked in either a teaching (n=78) or public hospital (n=99) while the rest worked in a general hospital (n=36), private (n=19) hospitals, oncology hospitals (n=22) and pediatric hospital (n=18)

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with the rest of respondents ticking other (n=13). For this question, participants were given the chance to select only one answer.

Knowledge about in-vivo gene therapy

Respondents were asked to evaluate and rate from 1 (meaning no knowledge) to 5 (meaning high knowledge) their knowledge of GTMP. The majority of respondents had limited knowledge of in-vivo gene therapy medicines, with only 9% (n=19/216) having strong knowledge of the subject.

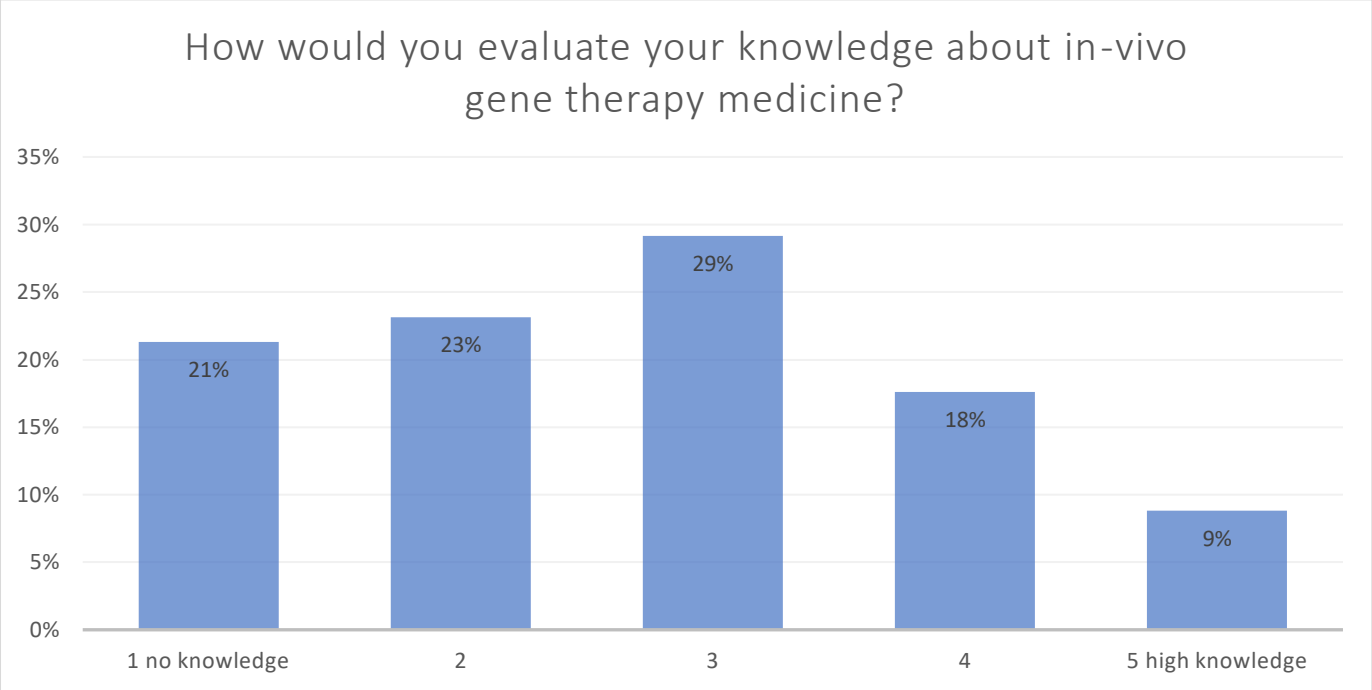

Figure 2: Percentage of responses (n=216) to question 3 "How would you evaluate your knowledge about in-vivo gene therapy medicine?"

For this question, 40 % (n=87/216) of the respondents were not ready or prepared for handling GTMP, with only 9% (n=19/216) of participants stating they have a high readiness/preparedness (Figure 3).

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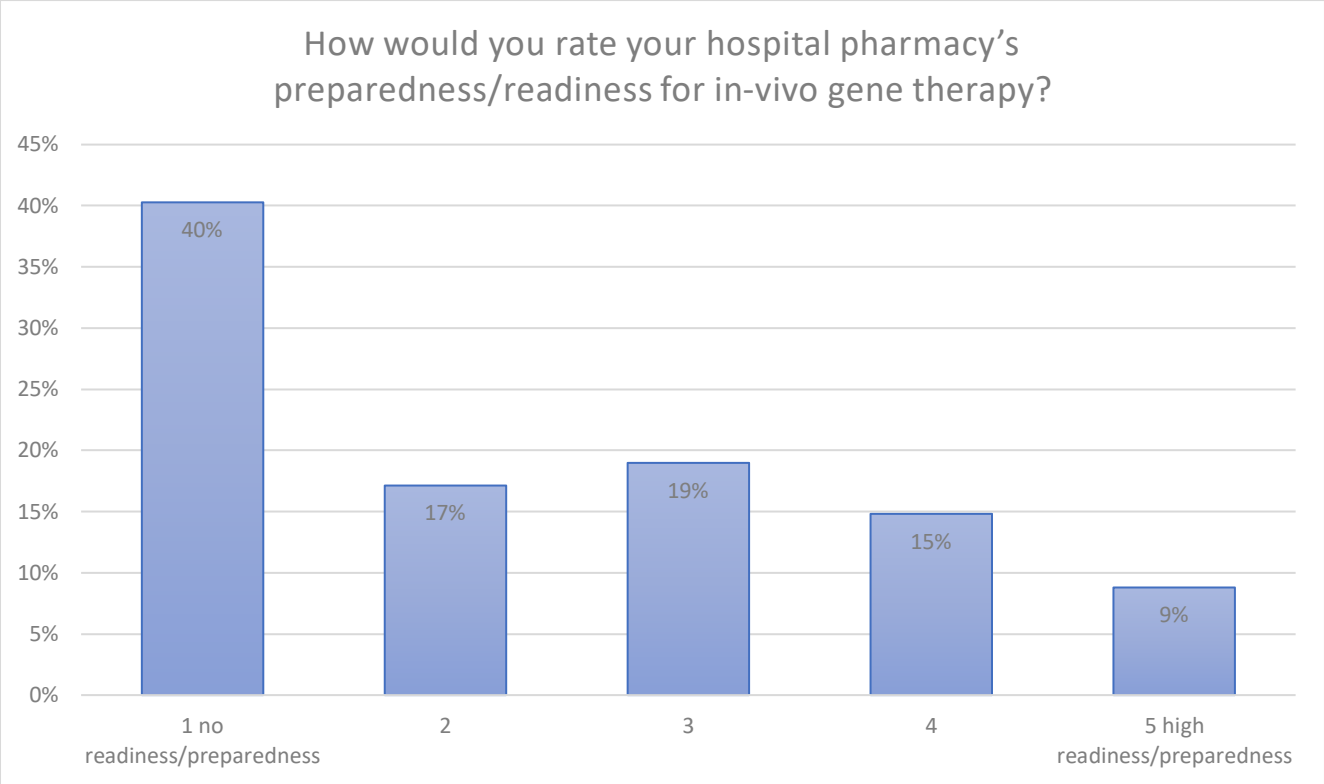

Figure 3: Percentage of responses (n=216) to question 4 "How would you rate your hospital pharmacy's preparedness/readiness for in-vivo gene therapy?"

Does your hospital provide in-vivo gene therapy?

When asked if their hospital provides GTMP 37% (n=79/216) of the respondents answered "yes" while 56% (n=120/216) answered that they do not. In addition, 8% (n=17/216) of respondents answered that they don't know (Figure 4).

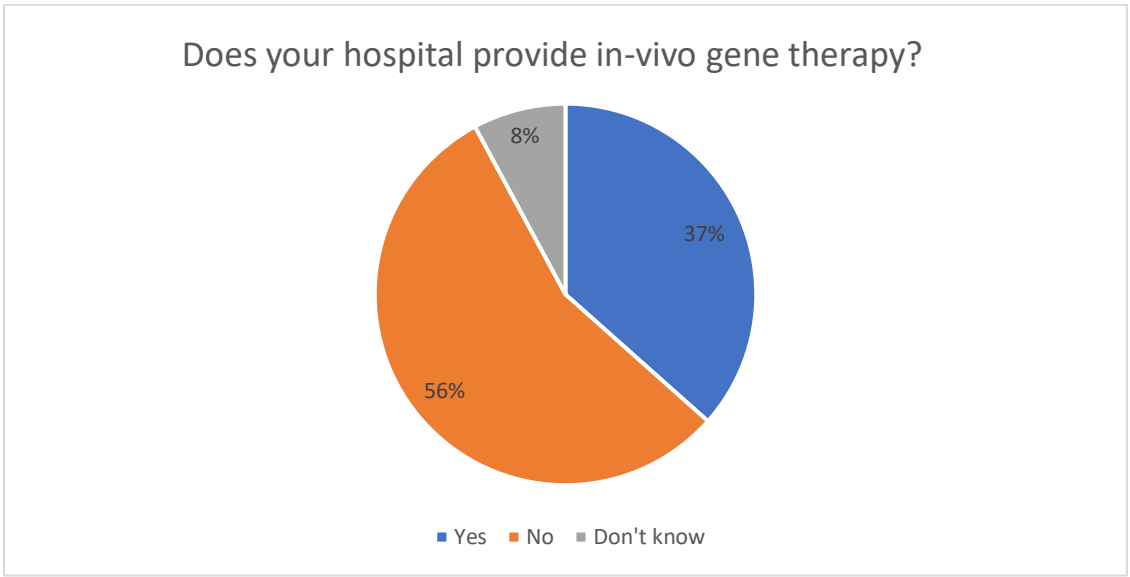

Figure 4: Percentage of responses (n=216) to question 5 "Does your hospital provide in-vivo gene therapy?"

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Hospitals providing in-vivo gene therapies

The remaining survey questions were divided between respondents that provide GTMP and the ones that do not or do not know. Even though 78 respondents answered that their hospital provides GTMP only 68 continued the survey and answered the following questions.

The first question for respondents providing GTMP was about the type of treatment provided in their hospitals. Respondents were able to tick more than one treatment. About three-quarters (74% (n=50/68)) of the respondents' hospitals provide clinical trial treatment with GTMP and 87% (n=59/68) offer standard treatment with approved medicines or medicines in early access programs (Figure 5).

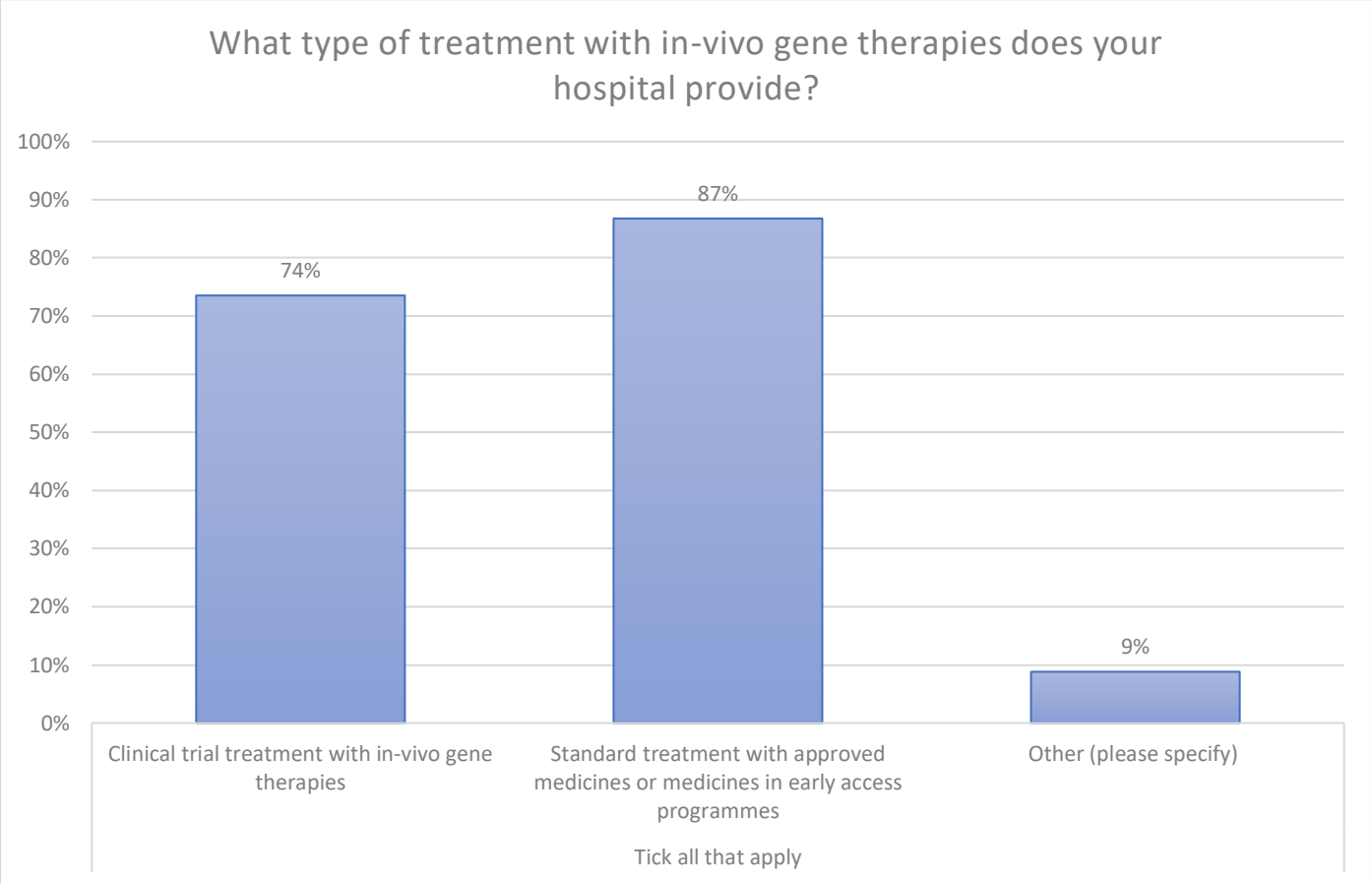

Figure 5: Percentage of respondents (n=68) that answered to the question 6 "What type of treatment with in-vivo gene therapies does your hospital provide?" (tick all that apply question)

To better understand the kind of GTMP that were provided by these hospitals, the questionnaire captured what agents were prepared; *Group 1 biological agent* (one that is unlikely to cause human disease to employee) or *Group 2 biological agent* (one that an cause human disease and might be a hazard to employees, although it is unlikely to spread to the community and in respect of which there is usually effective prophylaxis or treatment available).[11]

[11] *Directive 2000/54/EC of the European Parliament of 18th of September 2000*)

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Participants were able to tick both options. Group 1 biological agents were used by 74% (n=35/68) of respondents whereas only 38% use group 2 (n=26/68) biological agents.

Safety measures and preparations

GTMP require the set-up of safety measures as well as a protected and prepared environment for their handling. In total, 37% of hospitals providing GTMP have set up a biosafety committee or equivalent that oversees biological safety of the GTMP (Figure 6).

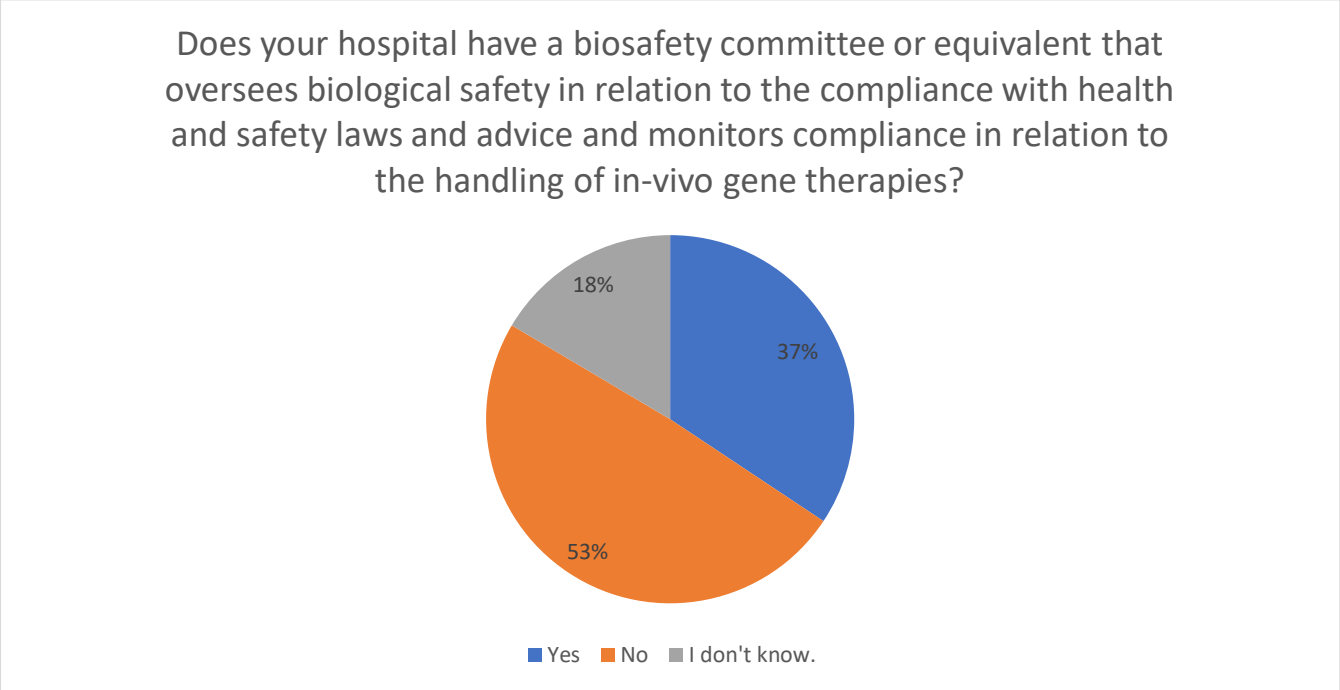

Figure 6: Percentage of respondents (n=68) that answered to the question 7 "Does your hospital have a biosafety committee or equivalent that oversees biological safety in relation to the compliance with health and safety laws and advice and monitors compliance in relation to the handling of in-vivo gene therapies?"

Moving on to the preparation of GTMP, the survey ascertained the availability of a clean room used for aseptic preparation for these therapies. One-third of the respondents (29%(n=20/68)) answered "yes" and that the negative pressure clean room is exclusively dedicated for GTMP (Figure 7).

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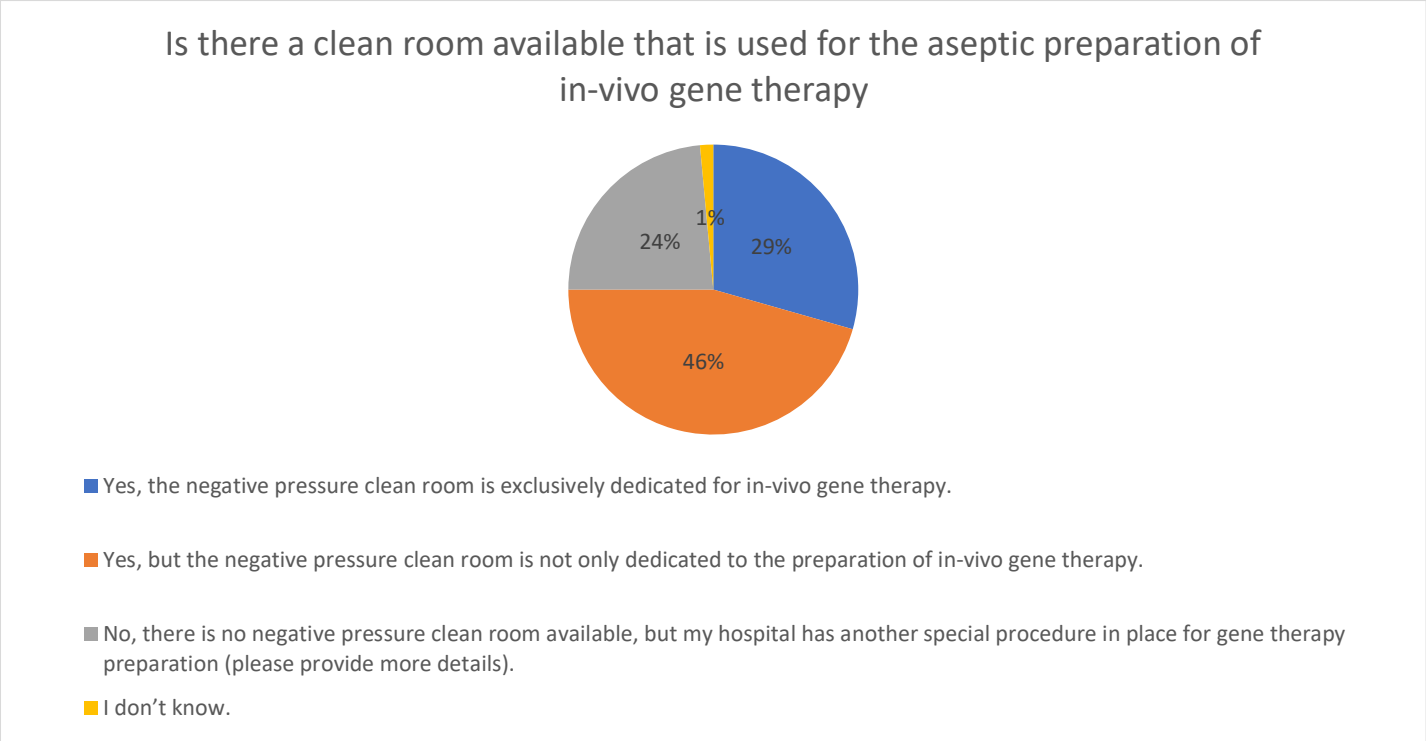

Figure 7: Percentage of respondents (n=68) that answered to the question 8 "Is there a clean room available that is used for the aseptic preparation of in-vivo gene therapy"

To investigate the preparation of the GTMP from these hospitals, the survey asked respondents where they handled their preparations. Most of the hospitals have a central clean room located in the hospital pharmacy 59% (n=40/62). (Figure 8).

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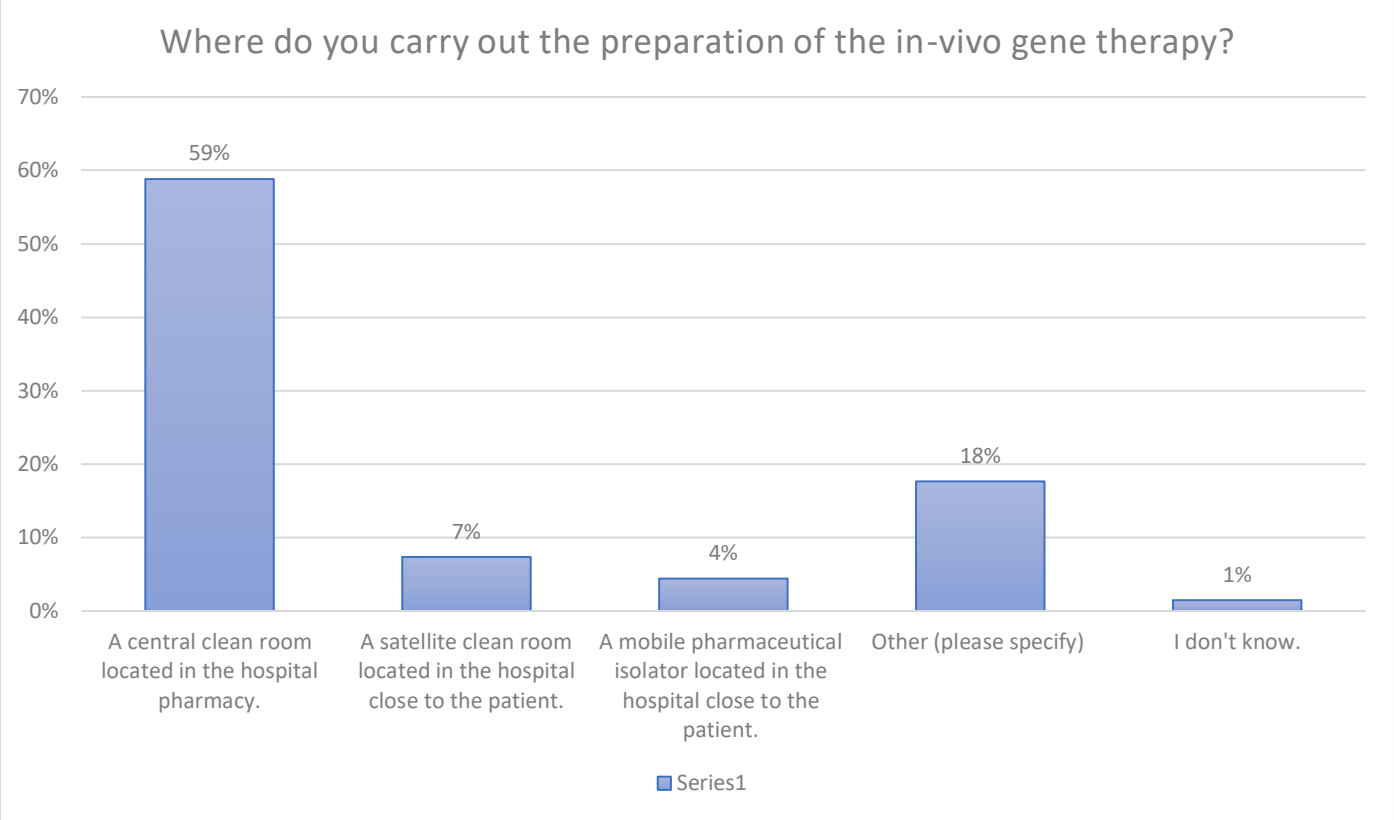

Figure 8: Percentage of respondents (n=68) that answered to the question 9 "Where do you carry out the preparation of the in-vivo gene therapy? …"

In addition, when the same respondents were asked what type of workstations are used for GTMP, 35% (n=24/68) answered that they use a biological safety cabinet class II B2 with 35% (n=24/68) using a biological safety cabinet class II A2 (Figure 9).

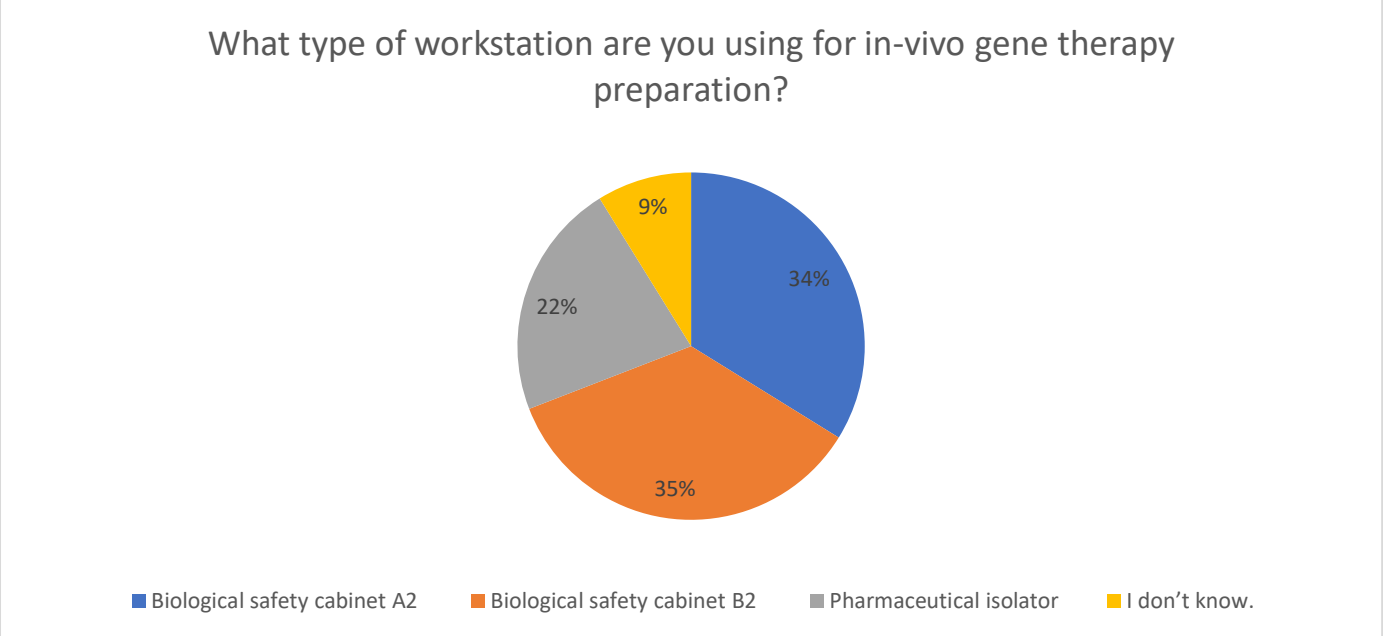

Figure 9: Percentage of respondents (n=68) that answered to question 10 "What type of workstation are you using for in-vivo gene therapy preparation?"

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Respondents explained that 81% (n=55/68) of their hospitals have Standard Operating Procedures (SOPs) in place regarding the handling of GTMP.

Storage facilities and waste management

Survey respondents were asked about the storage facilities their hospitals use to store gene therapy medicines and whether these are temperature monitored. Survey respondents were allowed to select all the options that applied to their hospitals. All respondents had a temperature monitoring system in place with 97% (n=66/68) of them having a fridge (2 to 8 °C) and 88% (n=60/68) having an ultra-freezer (- 80 to -60 °C) (Figure 10).

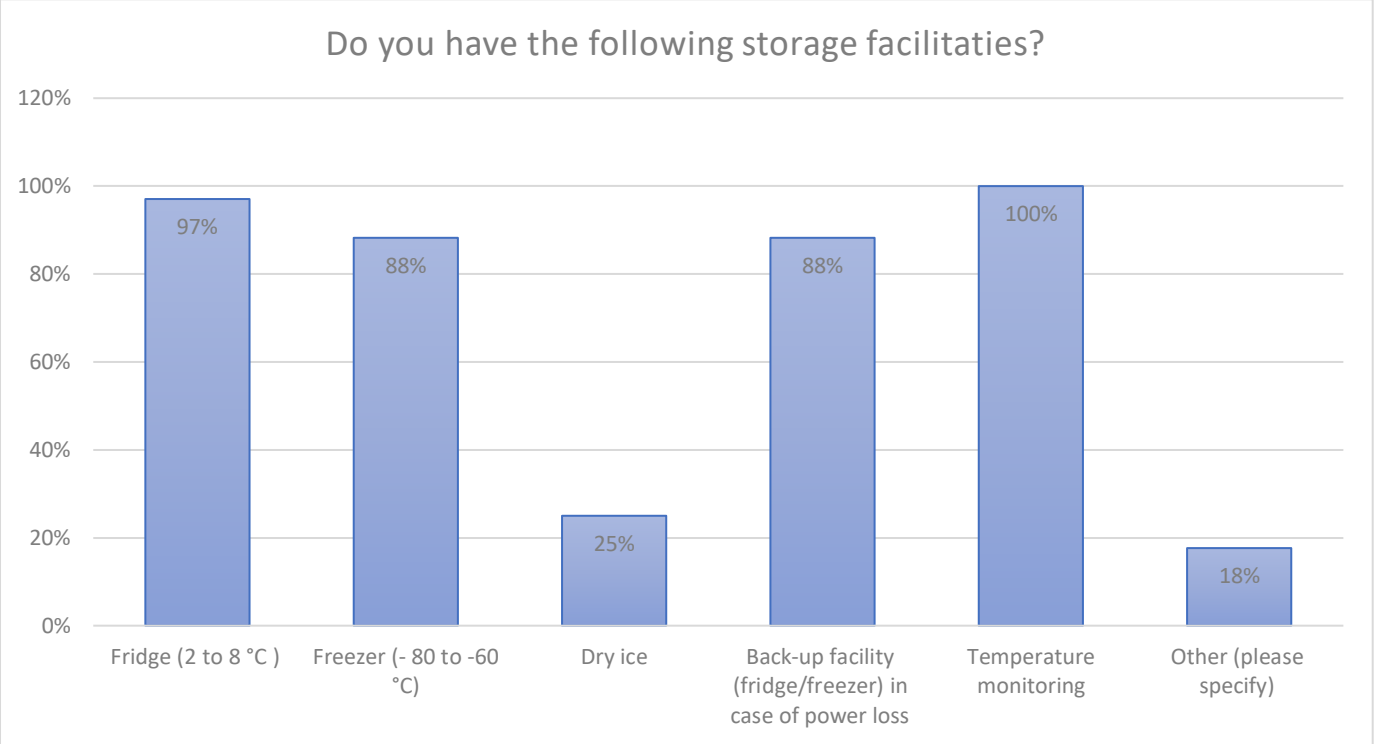

Figure 10: Percentage of respondents (n=68) that answered to the question 13 "Do you have the following the following storage facilities?" (tick all that apply question)

Most of the storage facilities in responding hospitals were located in the pharmacy department (84% (n=57/68)) and 7% (n=3/68) were located outside the pharmacy department (or the fridge was shared with other departments, or they didn't know).

To further investigate the pharmacy preparedness for GTMP, survey respondents had to answer and select from a list (multiple answers possible) of handling materials and procedures. A large majority of respondents (97%) stated that they have disinfectant agents and procedures for decontamination of work surfaces (92% (n=60/65). Only 57% (n=37/65) of the hospitals have a gene therapy specific spillage kit (Figure 11).

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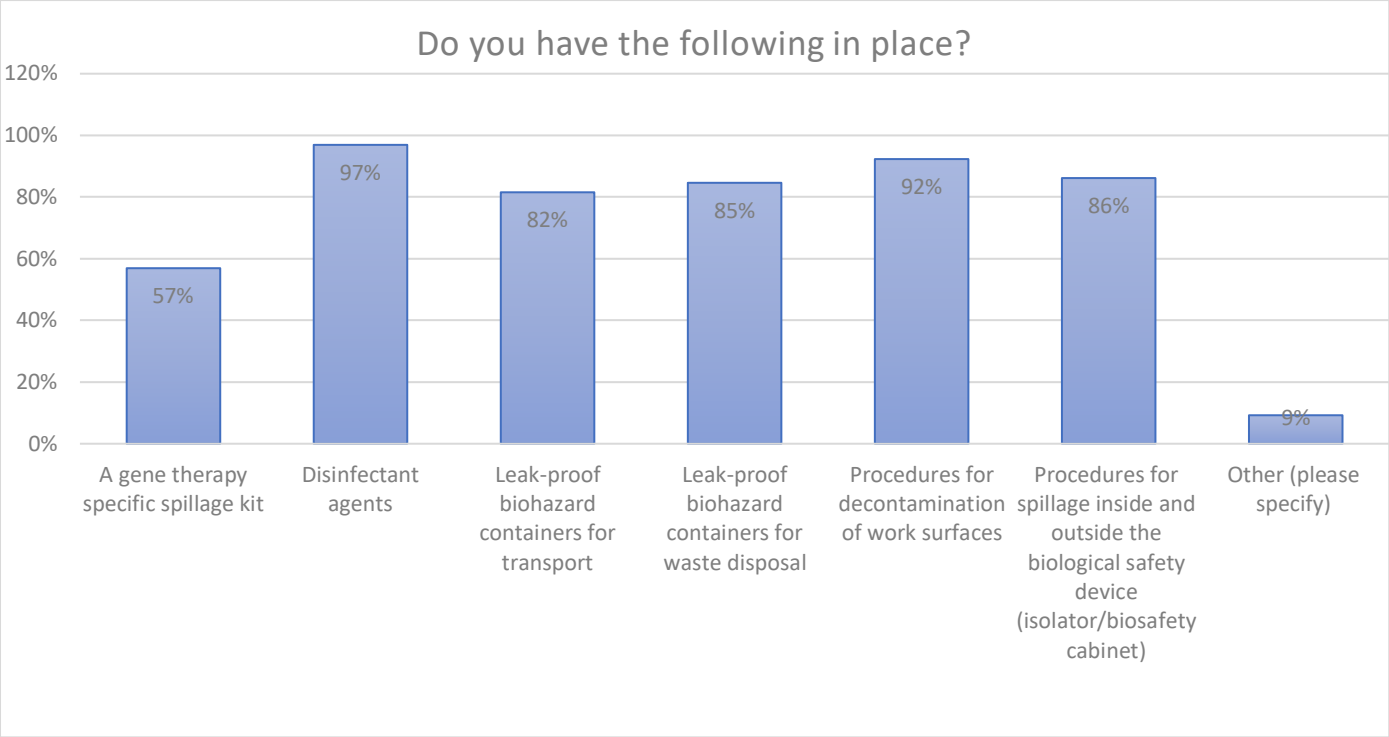

Figure 11: Percentage of respondents (n=65) that answered to the question 15 "Do you have the following in place? …" (thick all that apply question).

Survey respondents were given the chance to include other materials or procedures in place within their hospital and pharmacies when handling GTMP.

When asked about the disposal of biohazardous materials, 97% (n=63/65) of respondents answered that they do have a disposal for these materials while only 2% (n=1/65) answered that they do not. In addition, 43% (n=28/65) of the survey respondents have contractors with a special license to handle the GTMP waste while 35% (n=23/65) do not (Figure 12).

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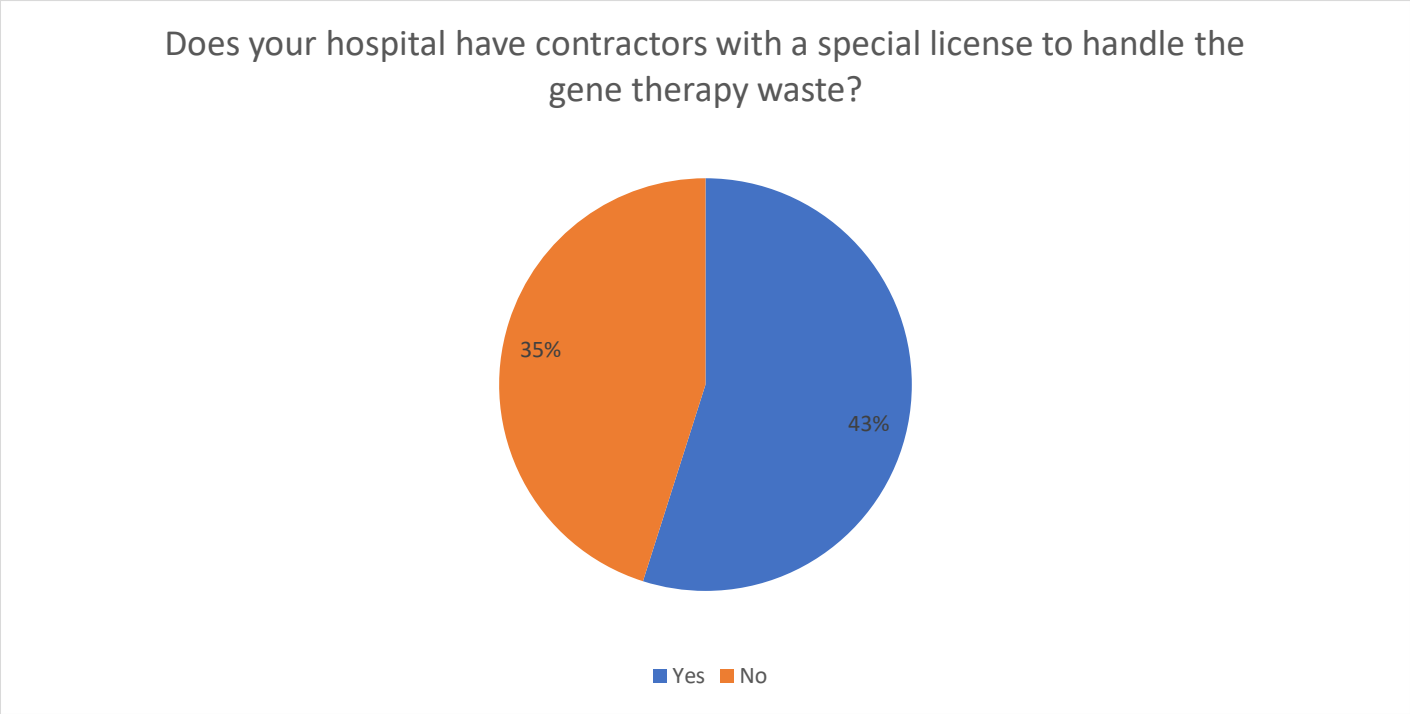

Figure 12: Percentage of respondents (n=65) that answered to the question 18 "Does your hospital have contractors with a special license to handle"

When asked about the waste management of GTMP, 12% (n=8/65) of the hospital pharmacies answered that they have an autoclave to handle waste while 5% (n=3/65) have an autoclave in the hospital but outside the pharmacy. In addition, 37% (n=24/65) answered that their hospital has an incineration facility that is used and "Others" with 45% (30/65) (Figure 13).

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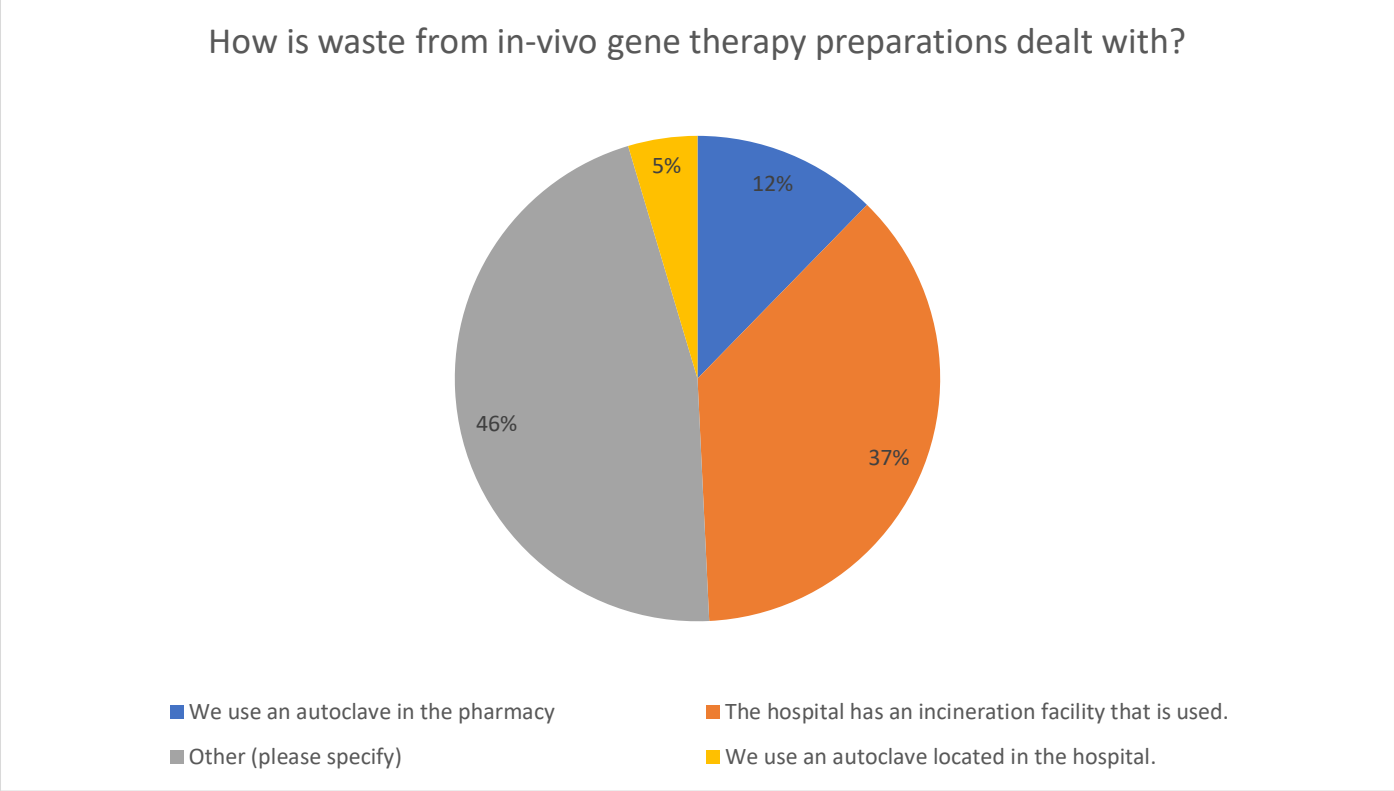

Figure 13: Percentage of respondents (n=65) that answered to the question 19 "How is waste from in-vivo gene therapy preparations dealt with?"

Education and training needs of the hospital pharmacies

The SIG was tasked to better understand the knowledge level of handling in-vivo GTMP and the training needs of hospital pharmacists. This was done with the aim for EAHP to develop tailored educational activities. The Survey asked participants to rate their knowledge level from 1 (no knowledge) to 5 (high knowledge). When asked about the general knowledge about genetics, most of the respondents answered 3 (meaning an average knowledge) with only a 5% (n=3/65) saying that they have no knowledge whatsoever (Figure 14).

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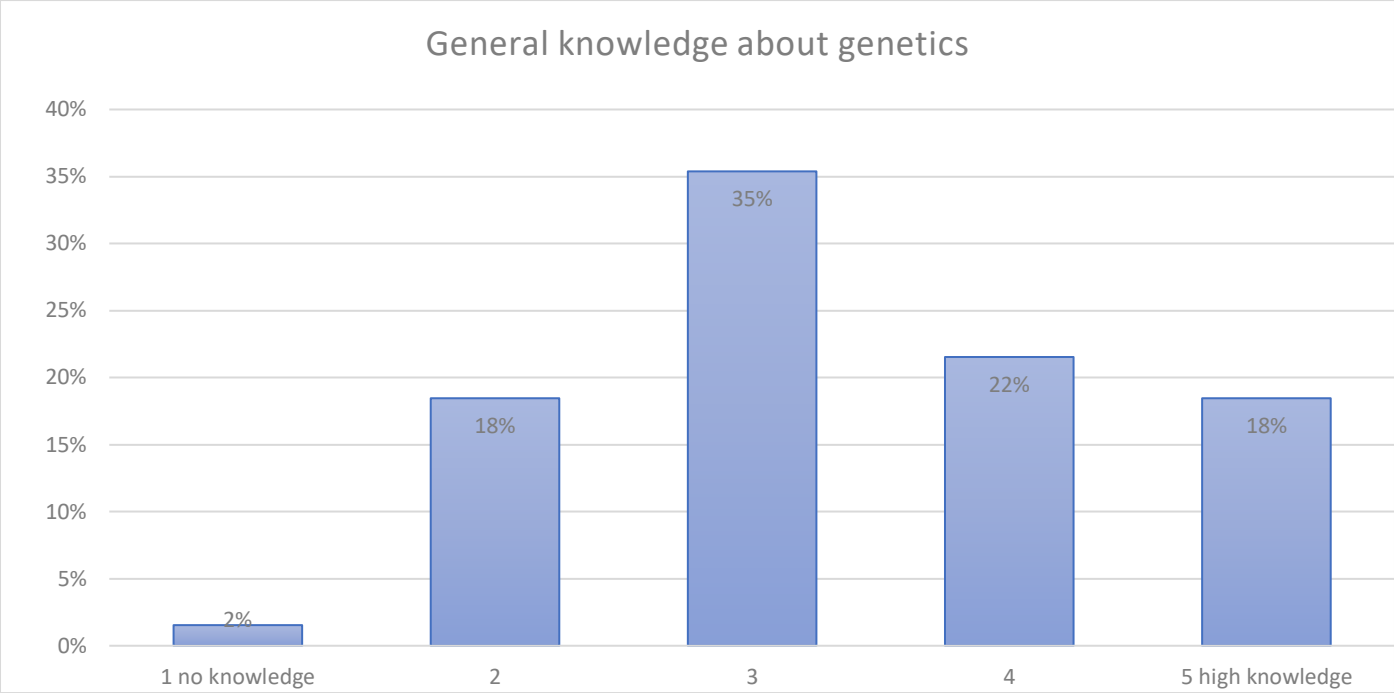

Figure 14: Percentage of responses (n=65) to the Question 20 "Rate your knowledge from 1 to 5 on…"

When asked about the specific knowledge about GTMP- principles, range of approaches (gene addition, gene editing, modulation of gene expression etc.) results are very similar to the previous question but with a slight increase of the hospital pharmacists having no knowledge (8% (n=5/65)) (Figure 15).

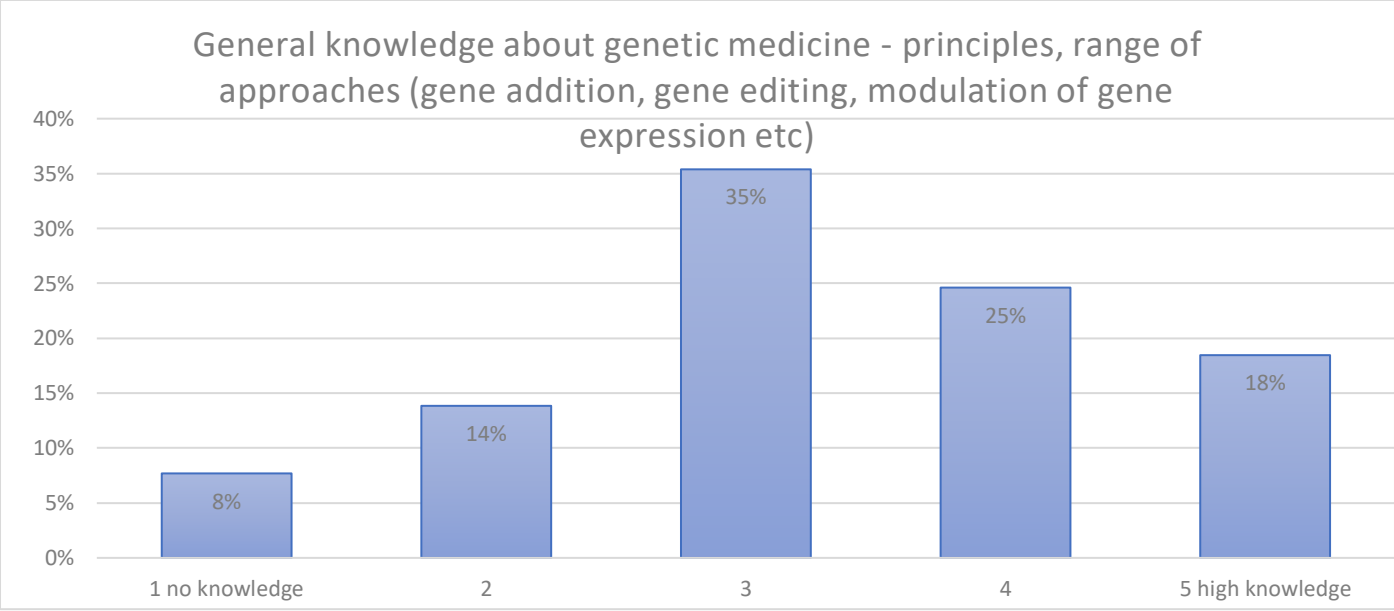

Figure 15: Percentage of responses (n=65) to the Question 20.b) "Rate your knowledge from 1 to 5 on…"

The survey then proceeded to ask about the knowledge of participants for different aspects of GTMP. The figures for all the questions have been included below. The areas where hospital pharmacists had less knowledge about are "In-vivo gene therapy vector design and engineering", "Pricing challenges, novel financing models" and "Requirements regarding long-term follow-up of the patients after receiving in-vivo gene therapy" (Figures 18, 19 and 26).

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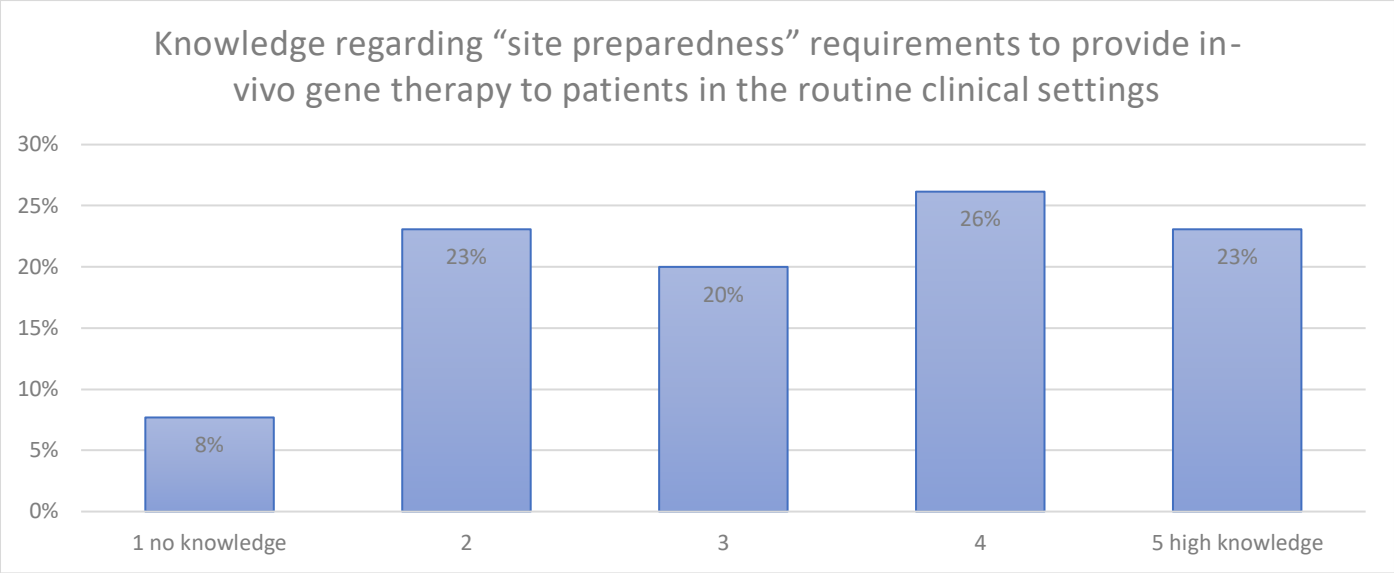

Figure 15: Percentage of responses (n=65) to the Question 20.c). “Rate your knowledge from 1 to 5 on…”

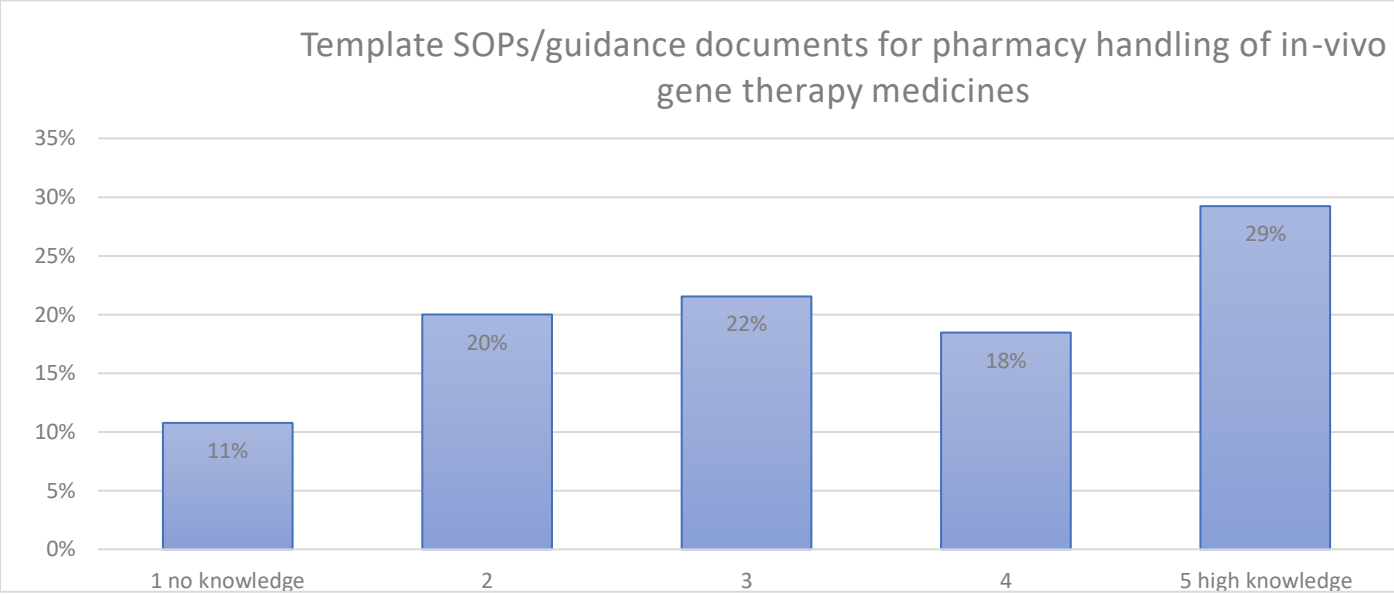

Figure 16: Percentage of responses (n=65) to the Question 20.d). “Rate your knowledge from 1 to 5 on…””

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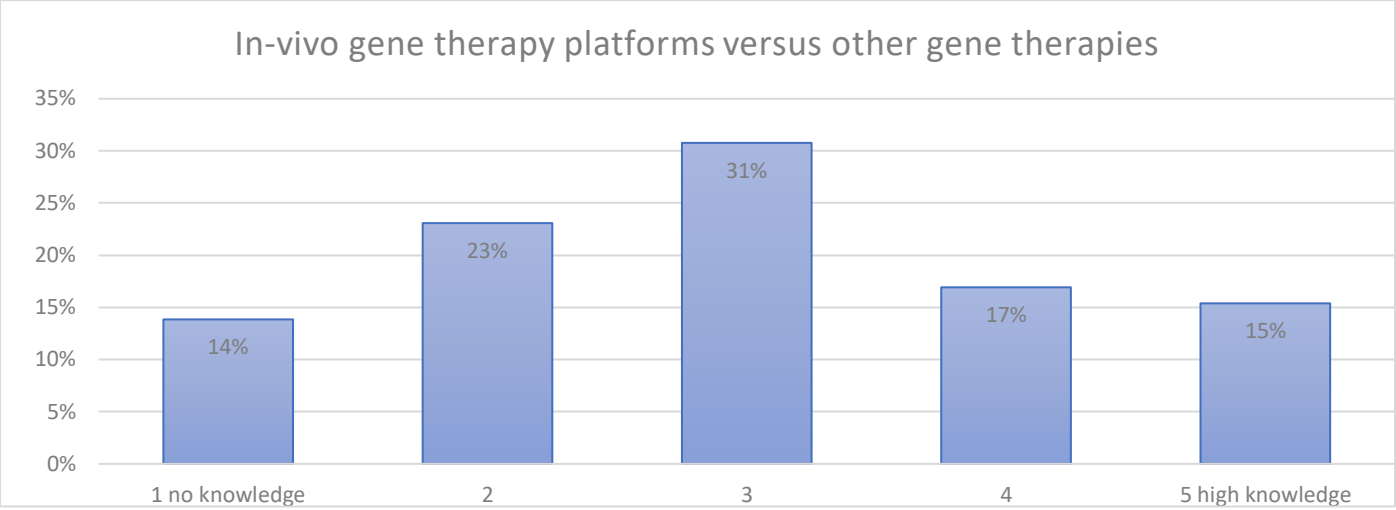

Figure 17: Percentage of responses (n=65) to the Question 20.e). "Rate your knowledge from 1 to 5 on…""

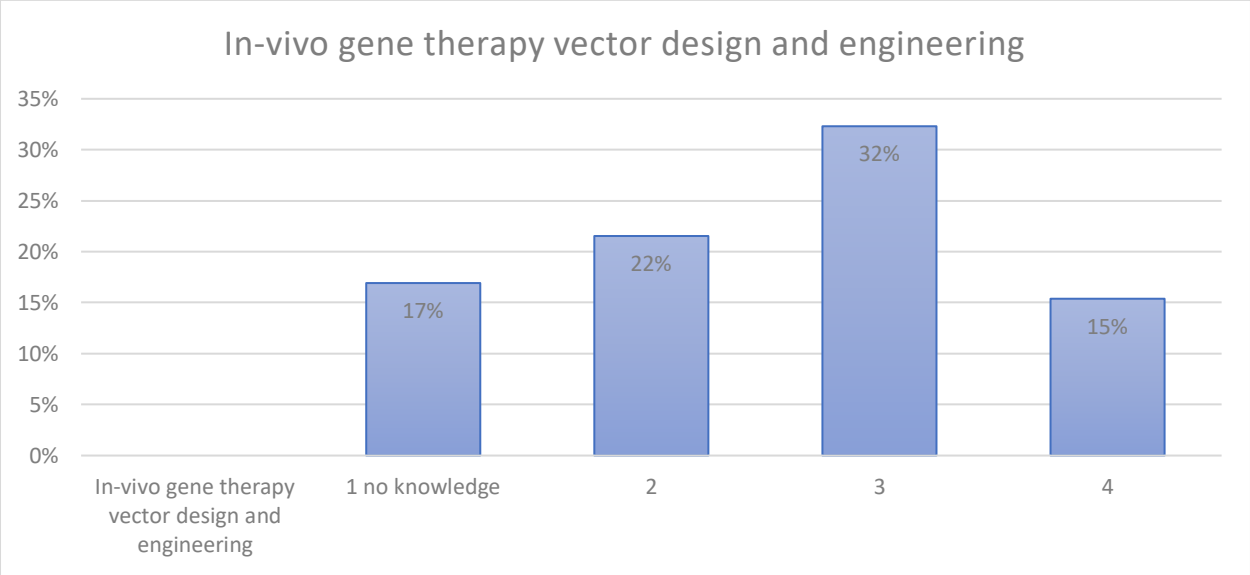

Figure 18: Percentage of responses (n=65) to the Question 20.f). "Rate your knowledge from 1 to 5 on…"

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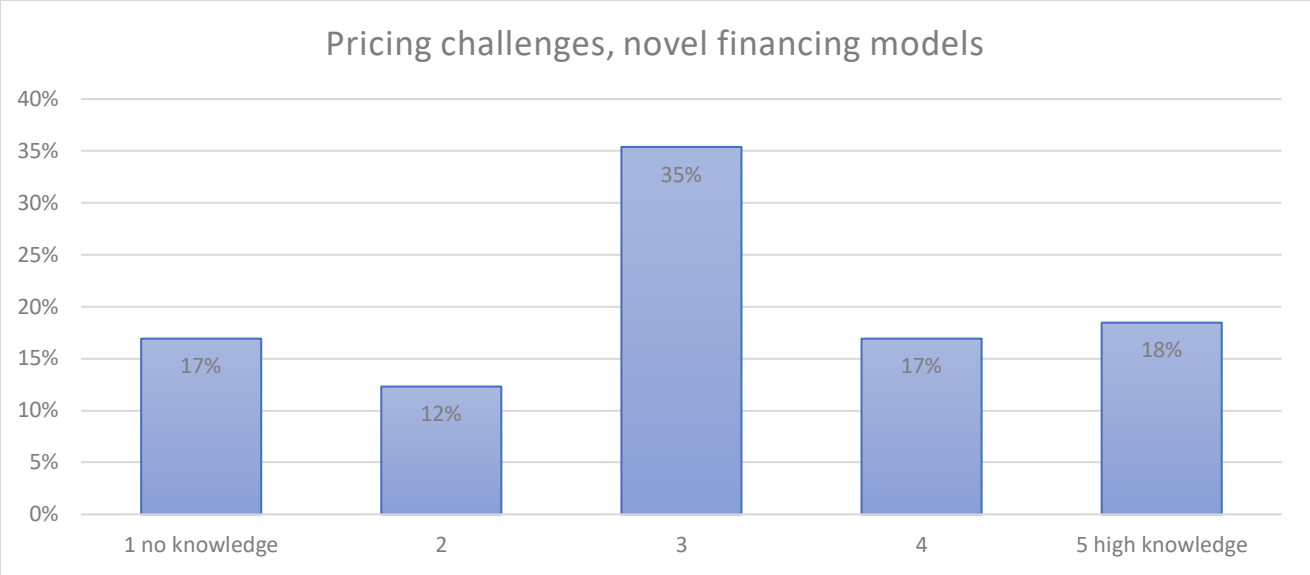

Figure 19: Percentage of responses (n=65) to the Question 20.g). "Rate your knowledge from 1 to 5 on…"

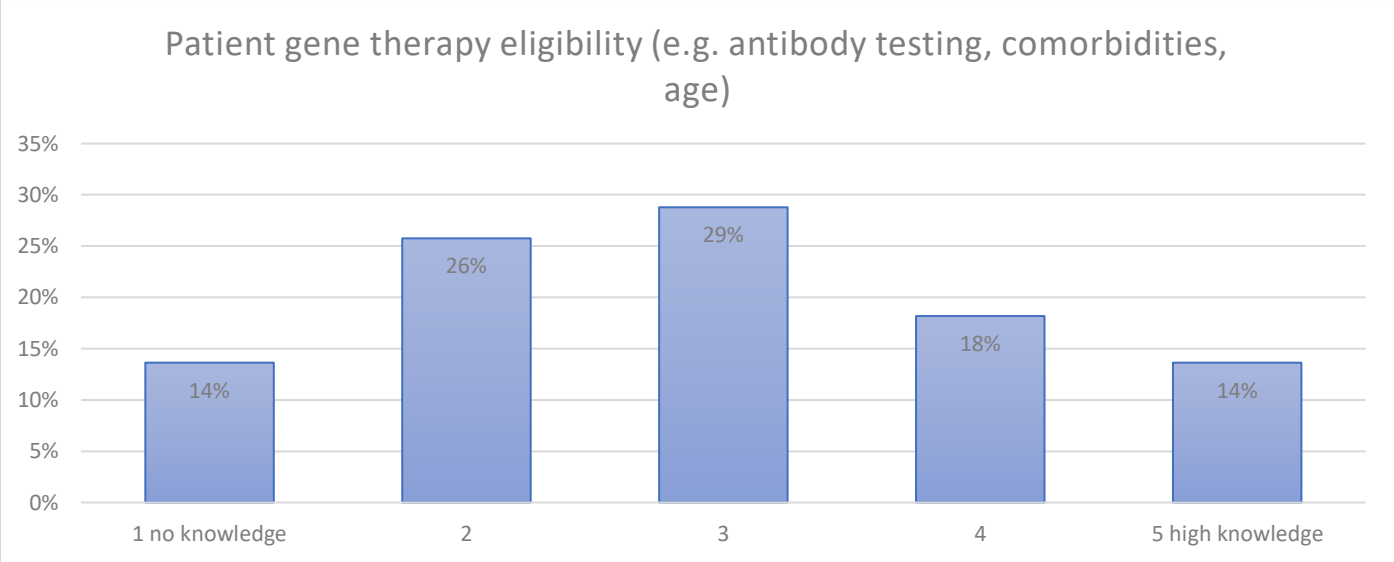

Figure 20: Percentage of responses (n=65) to the Question 20.h). "Rate your knowledge from 1 to 5 on…"

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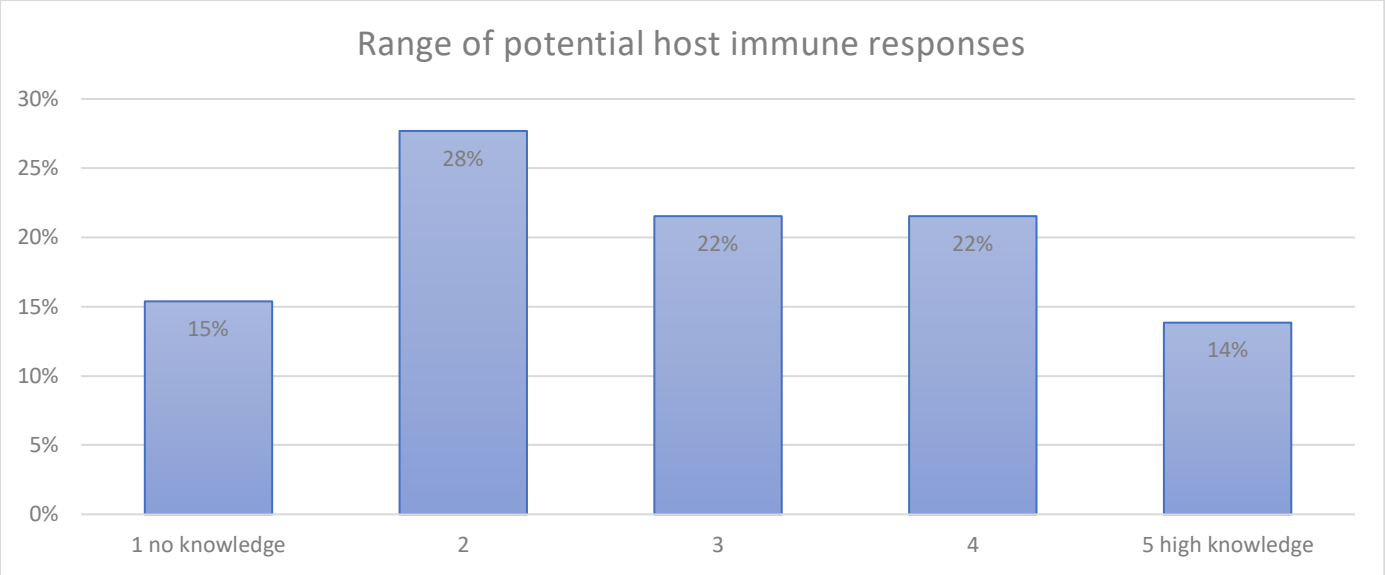

Figure 21: Percentage of responses (n=65) to the Question 20.i). "Rate your knowledge from 1 to 5 on…"

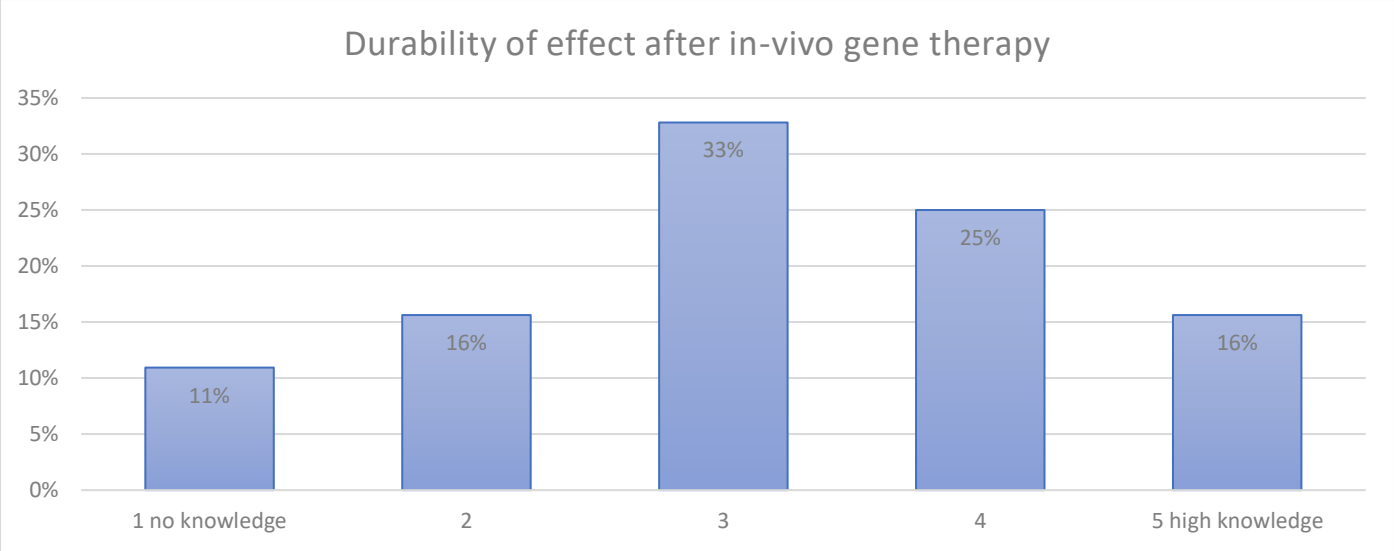

Figure 23: Percentage of responses (n=65) to the Question 20.j)." Rate your knowledge from 1 to 5 on…"

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Figure 24: Percentage of responses (n=65) to the Question 20.k).” Rate your knowledge from 1 to 5 on…””

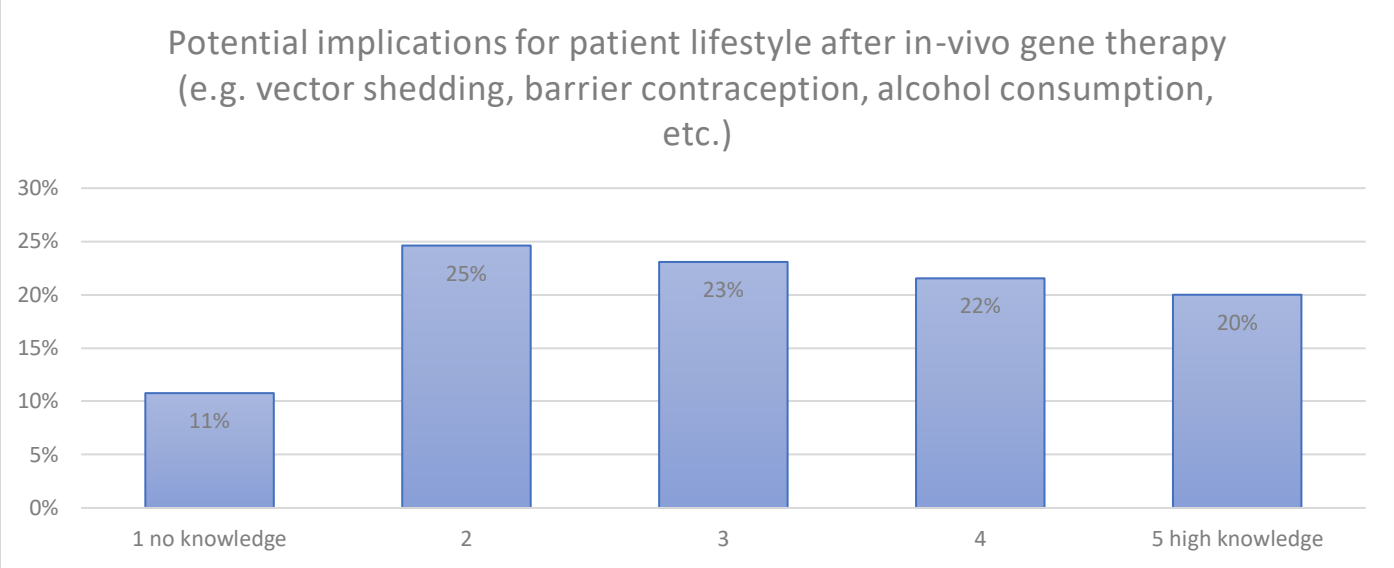

Figure 25: Percentage of responses (n=65) to the Question 20.l).” Rate your knowledge from 1 to 5 on…””

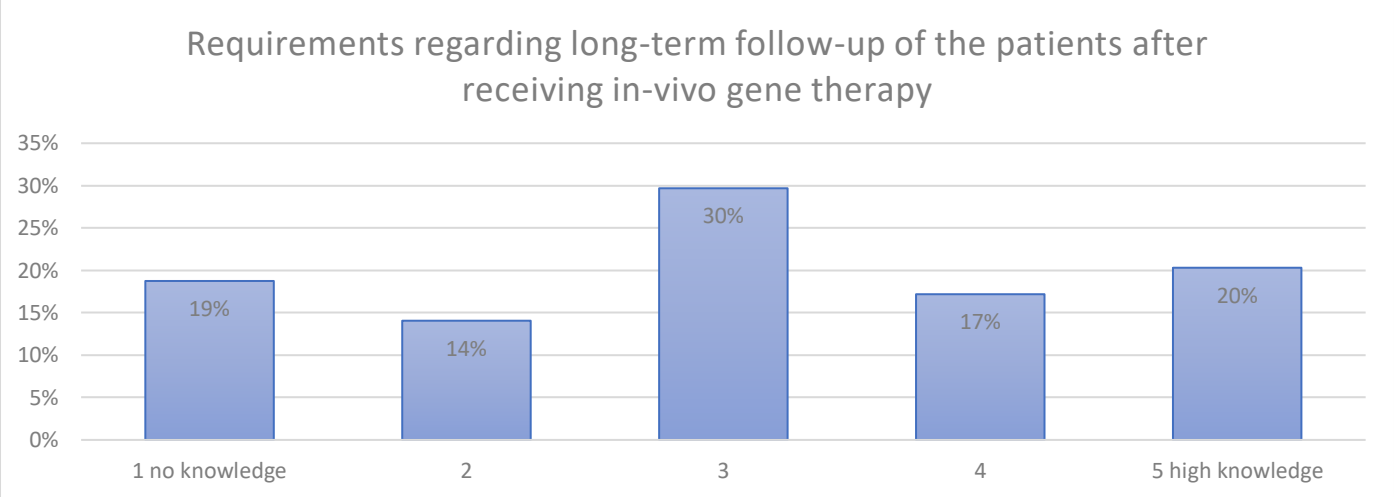

Figure 26: Percentage of responses (n=65) to the Question 20.m).

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In addition, 43% (n=28/65) of survey respondents confirmed that their countries/hospitals have guidance on how to handle GTMP. As explained in the introduction, there was a considerable difference in percentage of respondents from the United Kingdom (n=34), France (n=31) and Portugal (n=28). These countries have a high percentage of respondents thus the results for this question do not show a fully representative picture as some answers come from only a few of EAHP countries as seen below.

When asked which kind of materials or educational events could be most helpful for survey respondents, the large majority chose webinars (71% (n=46/65)) and e-learning courses (78%(n=53/65) (Figure 27).

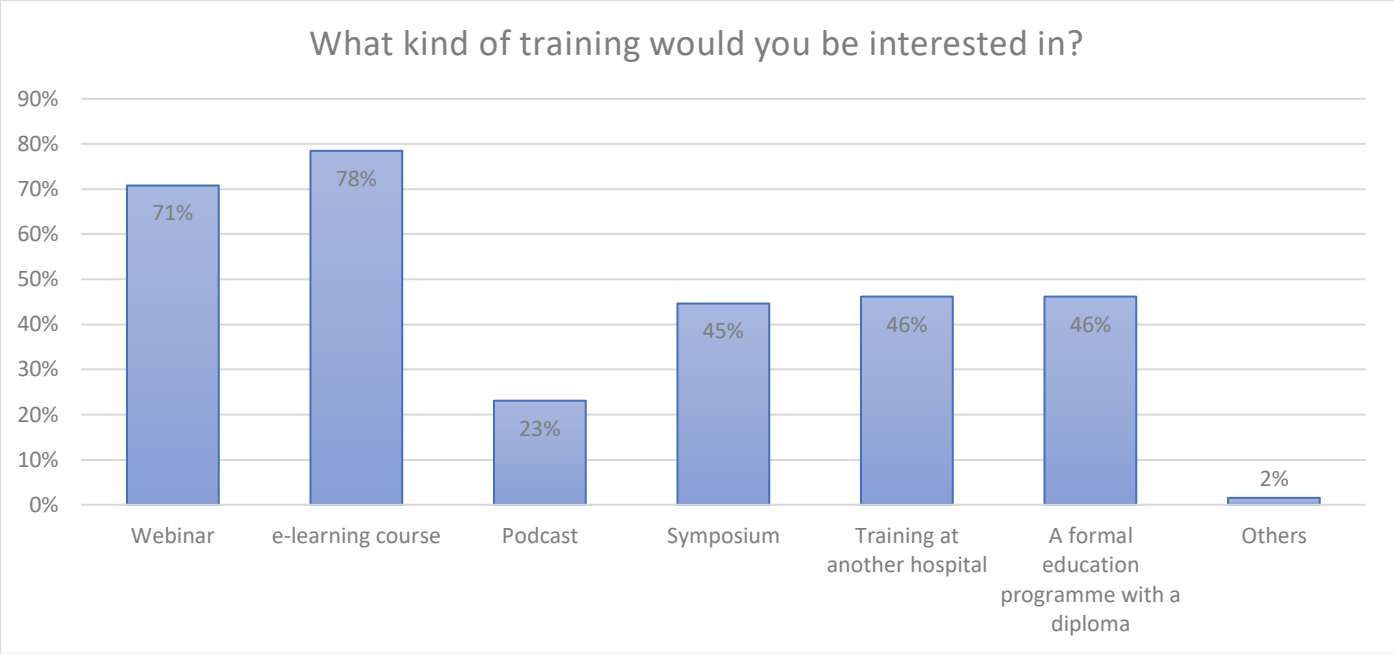

Figure 27: Percentage of responses (n=65) to Question 22 "What kind of training would you be interested in?"

Providing gene therapy

Financial approval for every patient is required in many countries/hospitals in Europe when providing GTMP. Financial approval was needed by 31% (n=20/65) of the Survey respondents' institutions. On the other hand, 20% (n=13/65) of the respondents do not need approval to provide these therapies.

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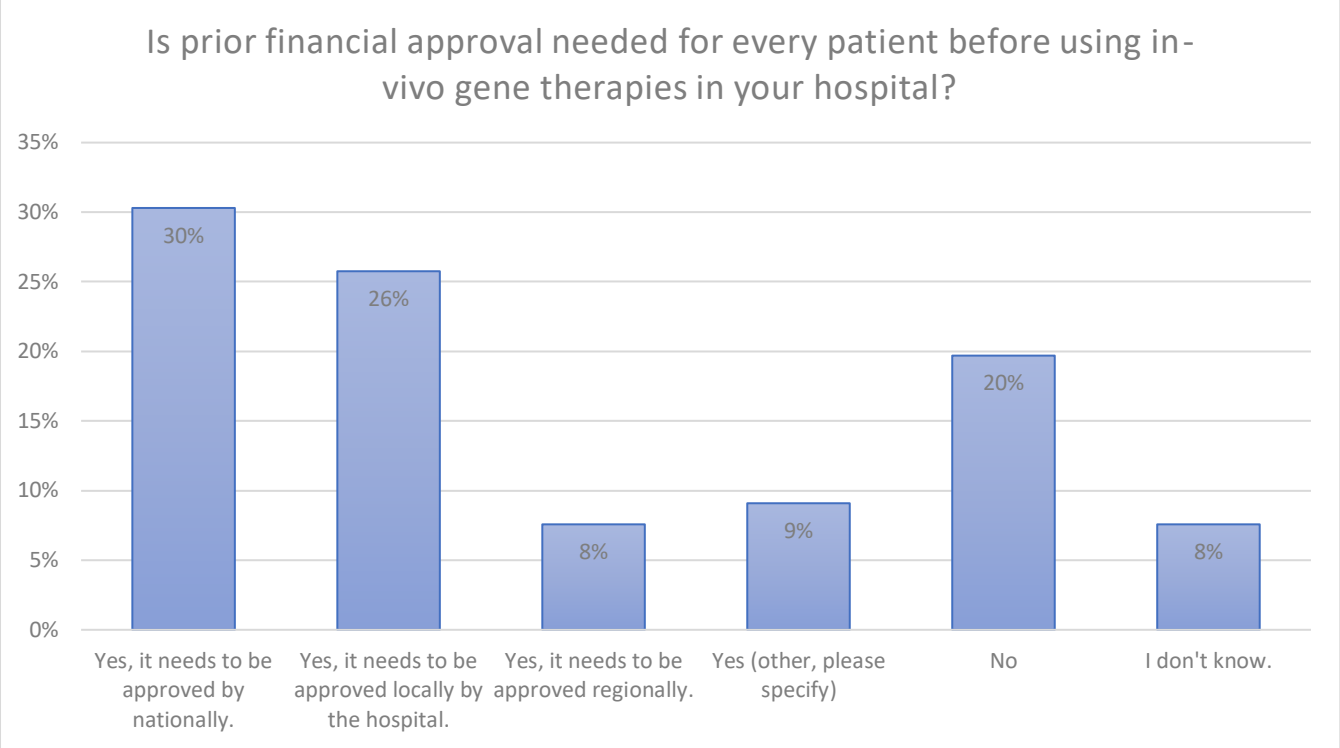

Figure 28: Percentage of responses (n=65) to Question 23 "Is prior financial approval needed for every patient before using in-vivo gene therapies in your hospital?"

A designated coordinator within pharmacy for the multidisciplinary hospital team working with GTMP was present in 60% (n=39/65) of the hospital pharmacies that completed the survey and provide in-vivo gene therapy. It is also worth noting that only 58% (n=38/65) of these pharmacy departments provide therapy specific training for staff handling GTMP (Figure 29).

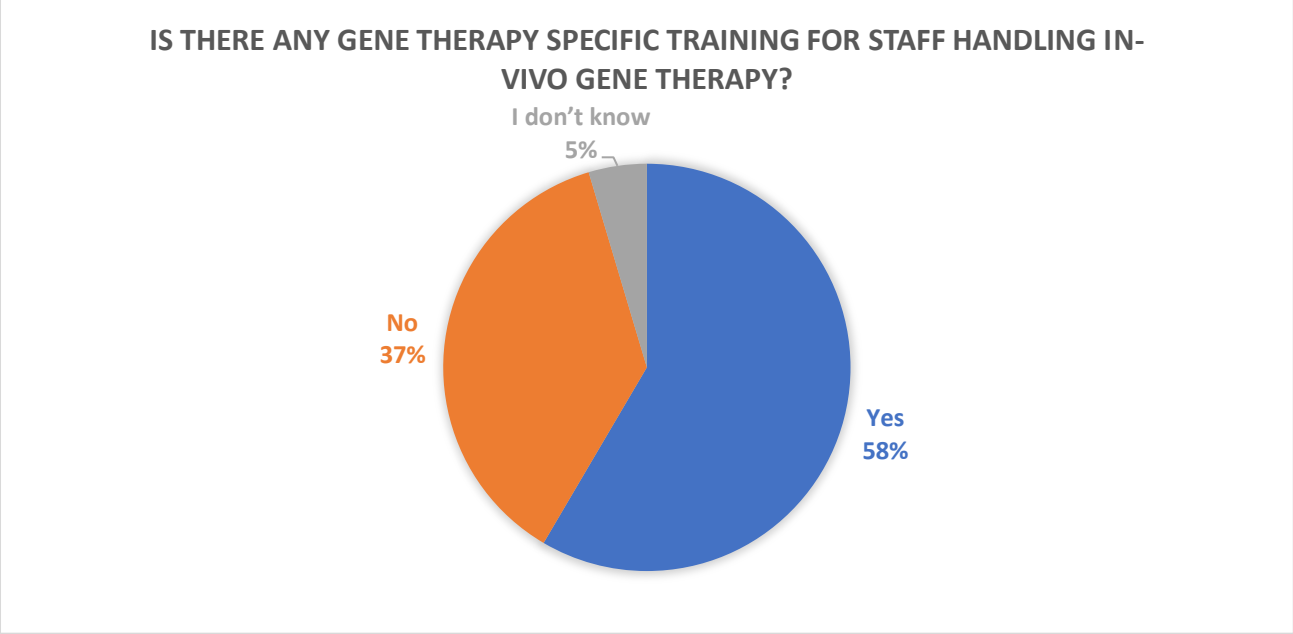

Figure 29: Percentage of responses (n=65) to the question 26 "Is there any gene therapy specific training for staff handling in-vivo gene therapy?"

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Survey participants where then asked if the occupational health (OH) department is involved in the procedures linked to GTMP. One-third (32% or n=21/65) answered "yes" while 43% (n=28/65) answered "no"; 25% (n=16/65) of the respondents answered that they didn't know (Figure 30).

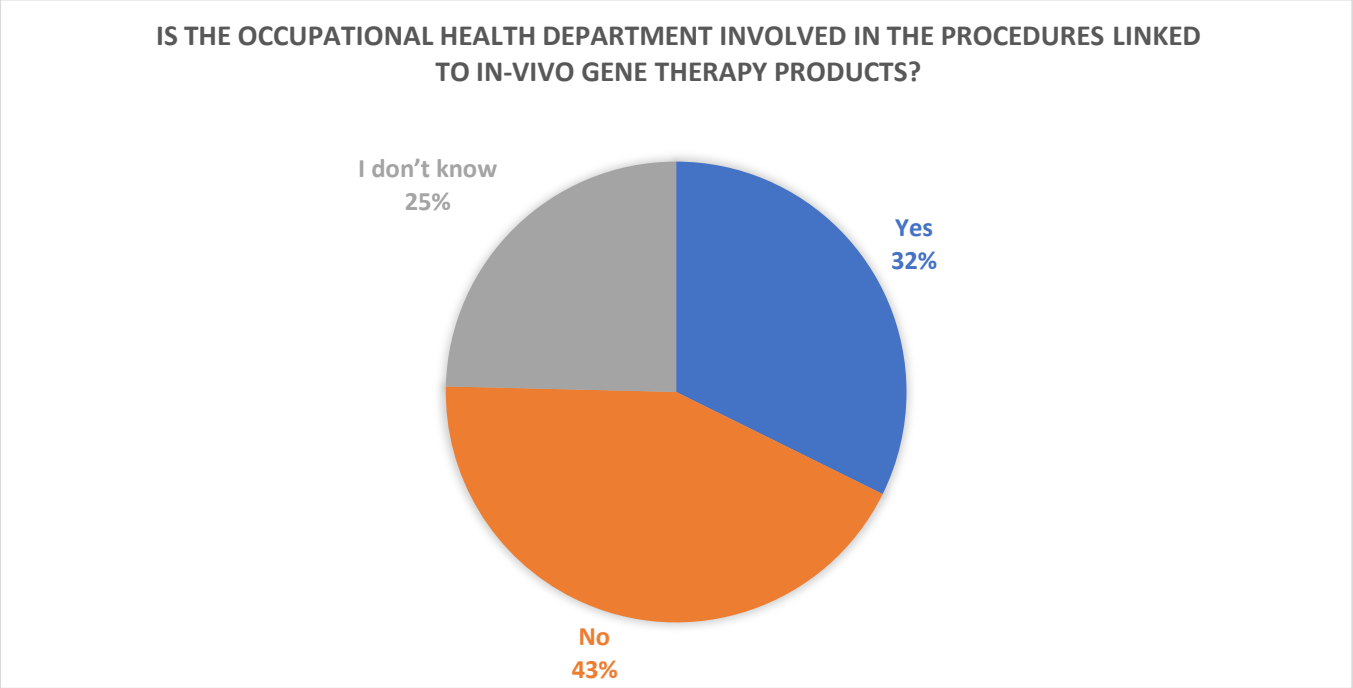

Figure 30: Percentage of response (n=65) to the question 29 "Is there any gene therapy specific training for staff handling in-vivo gene therapy?"

Hospital Pharmacists not providing gene therapy

The SIG also wanted to investigate the preparedness and status of hospital pharmacies that are not providing GTMP at the time the survey was open. The first question asked was whether these pharmacies are planning to provide GTMP in the future: 14% (n=16/116) answered that they are planning to provide GTMP within the next 2 years and 23% (n=27/116) will provide gene therapy but within the next 2-5 years; 40% (n=46/116) don't know if they will provide GTMP and 23% (n=27/116) will not provide GTMP.

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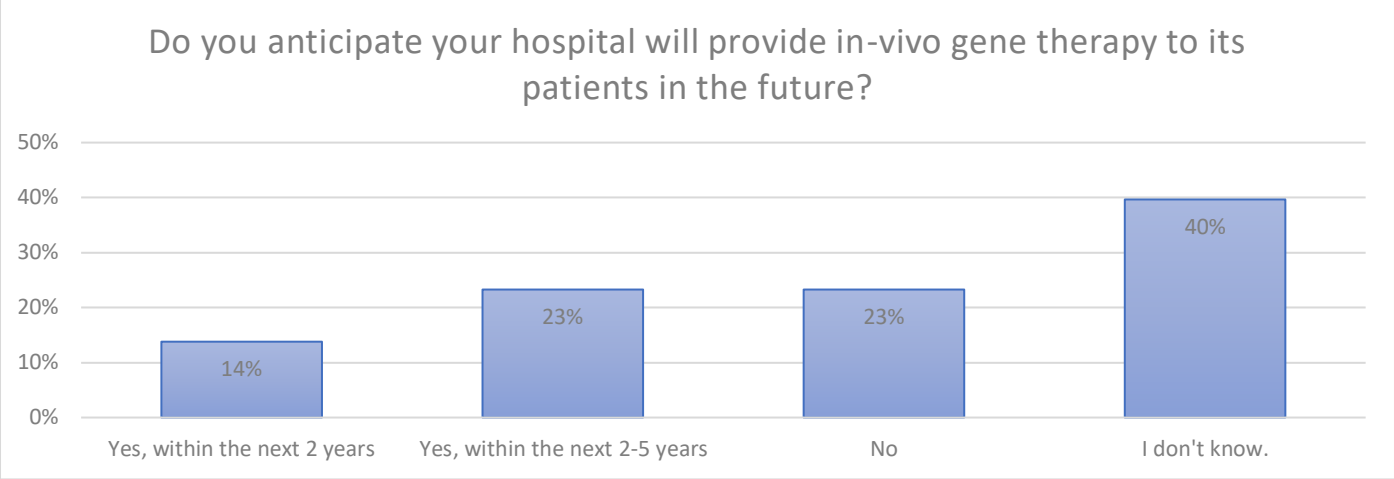

Figure 31: Percentage of response (n=116) to the Question 32 "Do you anticipate your hospital will provide in-vivo gene therapy to its patients in the future"

To further investigate about future GTMP provision, the Survey asked respondents on the type of treatments that the hospital pharmacies could provide: 38% (n=44/116) will provide clinical trial treatment with GTMP and 47% (n=63/116) will provide standard treatment with approved medicines or medicines in early access programs (Figure 32).

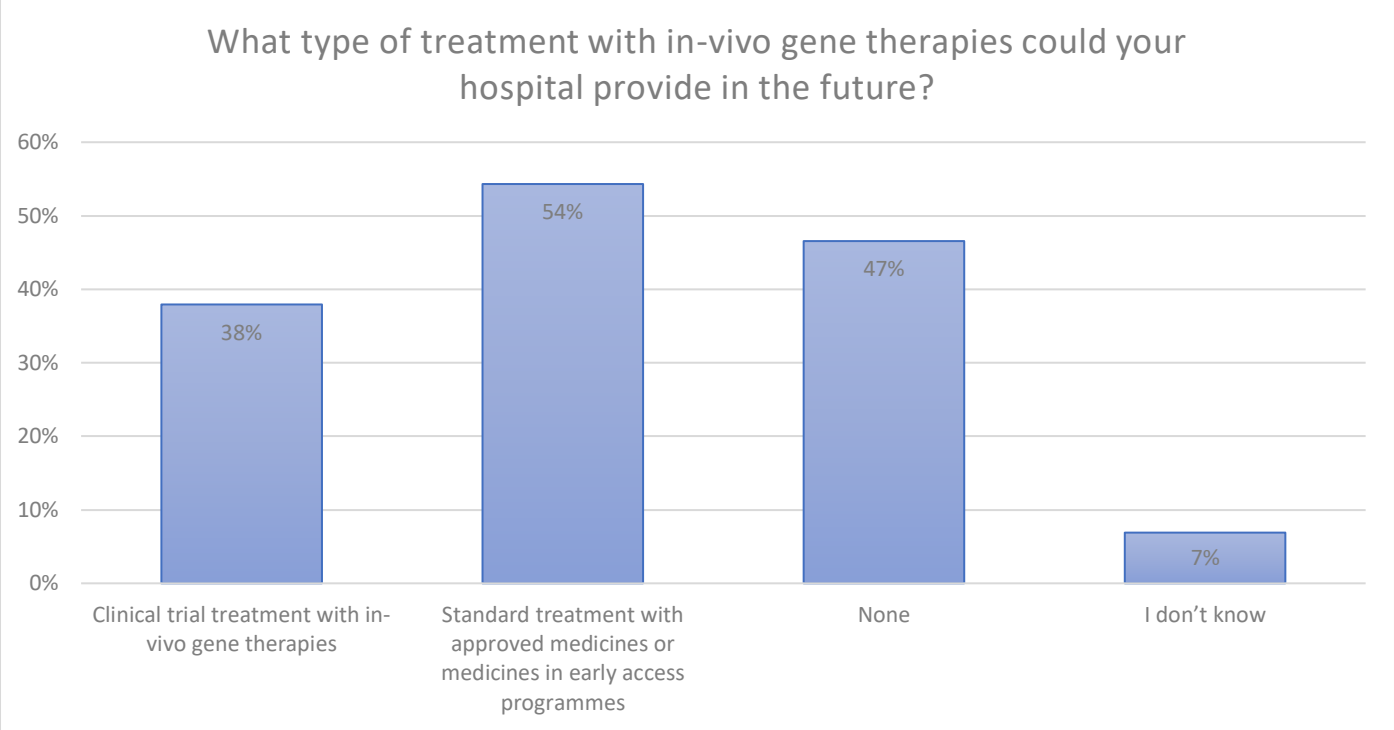

Figure 32: Percentage of responses (n=116) to the Question 33 "What type of treatment with in-vivo gene therapies could your hospital provide in the future?"

When asked about the kind of gene therapy that they could provide, 39% (n=45/116) provide group 1 Biological agent therapies and 13% (n=15/116) could provide group 2 Biological agents, while 58% (n=67/116) don't know the kind of gene therapies they could deliver.

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The SIG also asked the hospitals that do not currently provide GTMP if they have a biosafety committee or equivalent that could oversee biological safety in relation to the compliance with health and safety laws as well as advice and monitor compliance in relation to the handling of in-vivo gene therapies. Only 15% (n=17/116) of these hospitals have a Biosafety Committee while 67% (n=78/116). 18% (n=21/116) didn't know (Figure 33).

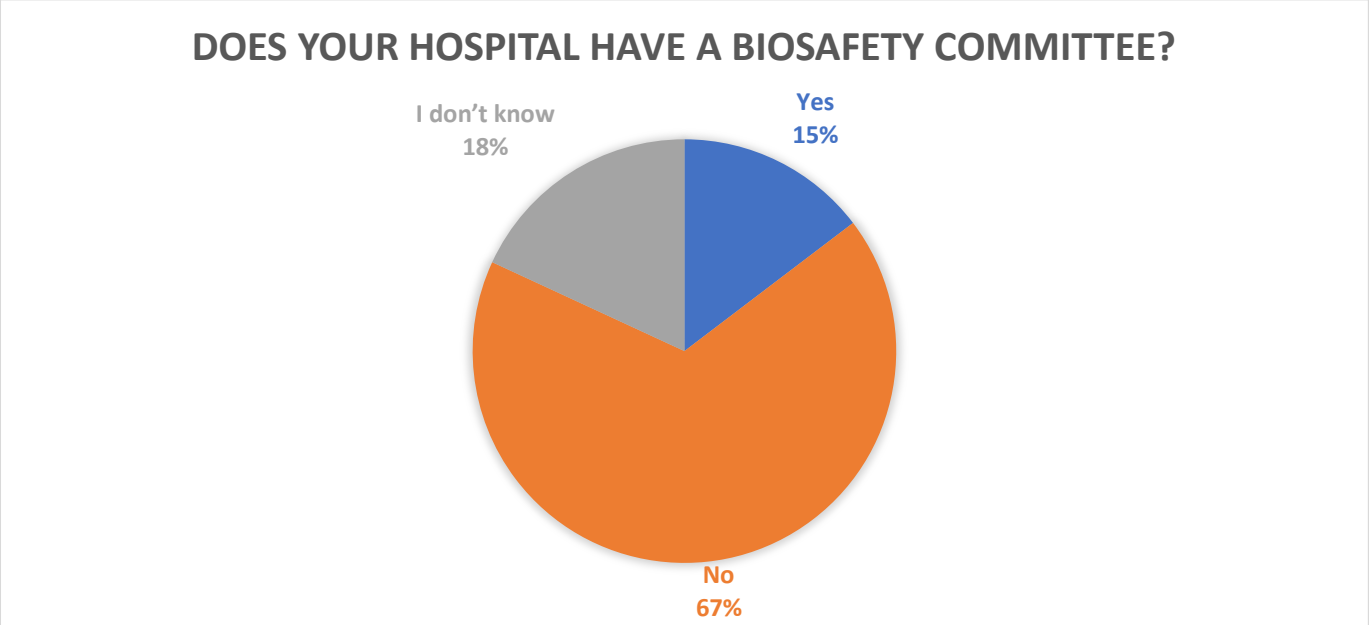

Figure 33: Percentage of responses (n=116) to the question 35 "Does your hospital have a biosafety committee?"

A total of 52% (n=60/116) of hospitals that currently do not provide GTMP had a central clean room located in the hospital pharmacy that could be used for the aseptic preparation of in-vivo gene therapy. Only 2% (n=2/116) had a mobile isolator located in the hospital close to the patient could be used for this purpose and 3% (n=4/116) had a satellite clean room located in the hospital close to the patient could be used (Figure 34).

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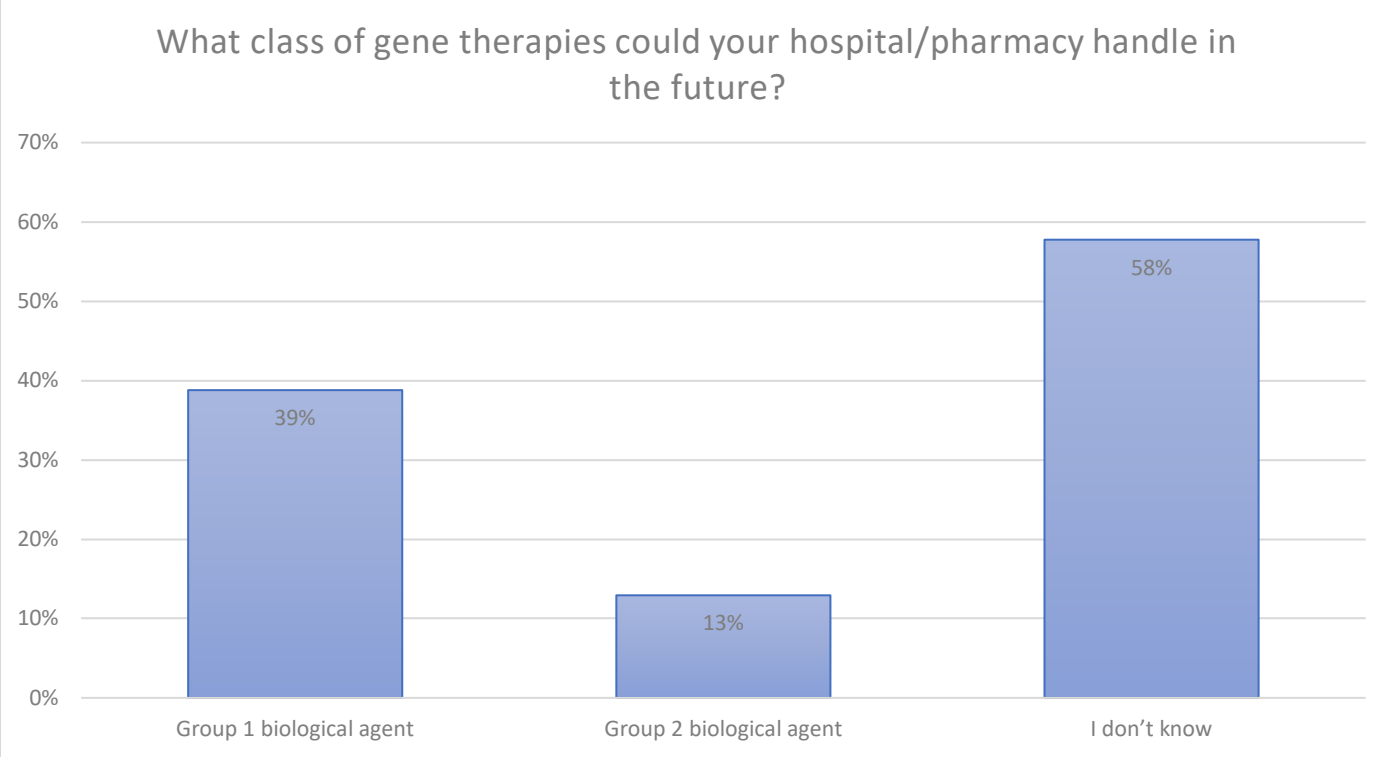

Figure 34: Percentage of response (n=116) to the Question 37: "Is there a clean room available that could be used for the aseptic preparation of in-vivo gene therapy?"

Responses in regard to storage facilities from hospitals currently not providing GTMP showed that 97% (n=112/116) have fridges (2 to 8 °C), 92% (n=107/116) have a temperature monitoring system and 48% (n=56/116) have an ultra-freezer (- 80 to -60 °C). In addition, 54% (n=63/116) have back-up facilities (fridge/ultra-freezer) in case of power loss or malfunction (Figure 35).

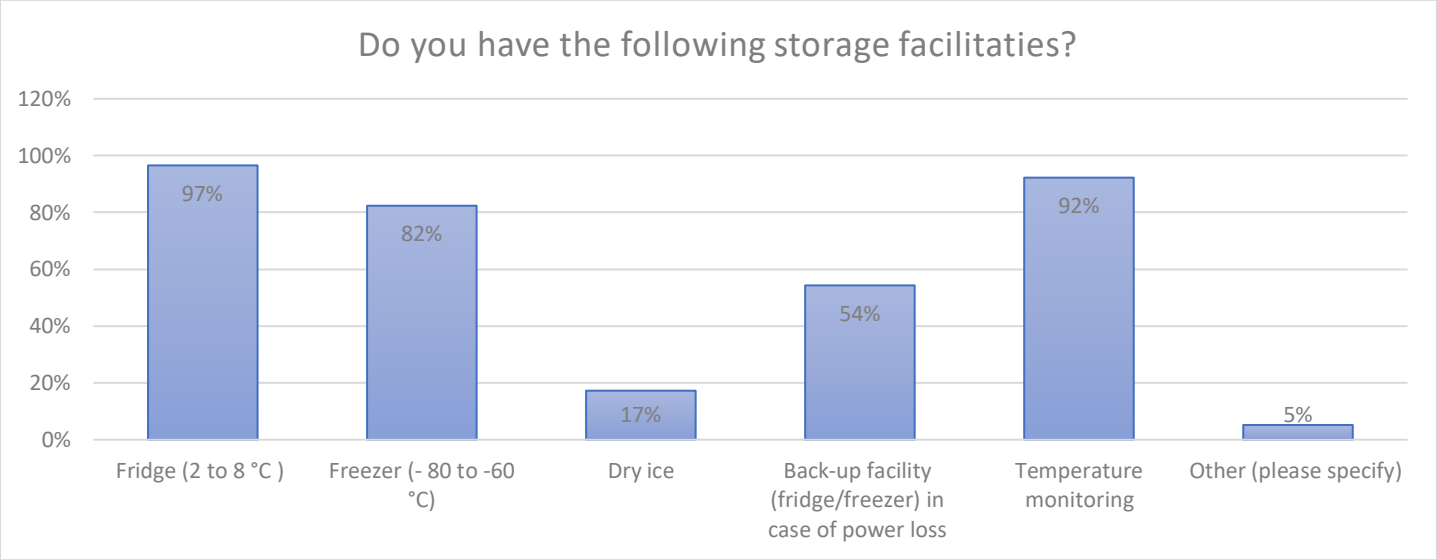

Figure 35: Percentage of response (n=116) to the Question 38: "Do you have the following storage facilities?"

The same respondents had to answer and rate their education and training needs in different aspects of handling preparation and delivery of GTMP. The biggest needs from these hospitals were for the "Knowledge regarding "site

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preparedness" requirements to provide in-vivo gene therapy to patients in the routine clinical settings" (44% (n=44/101), "Template SOPs/guidance documents for pharmacy handling of in-vivo gene therapy medicines" (43% (n=43/101)" and "In-vivo gene therapy platforms versus other gene therapies" (43% (n=43/101) (Figure 36).

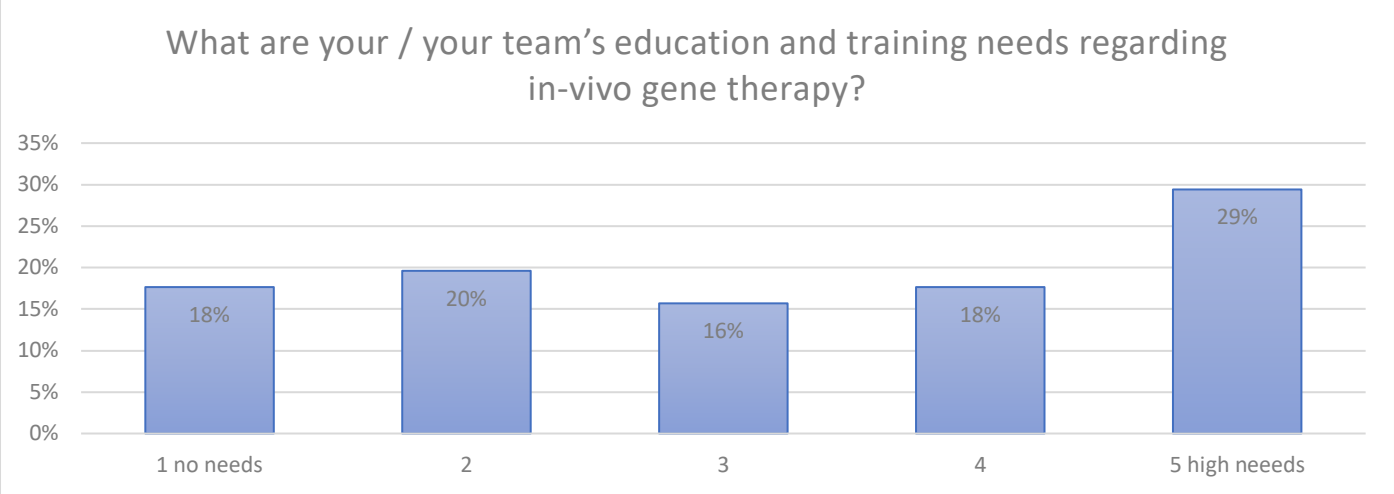

Figure 36: Percentage of responses (n=101) for the question 42.a)" Rate your education and training needs."

The rest of the questions for the training needs can be found as Appendix II. When these hospitals were asked what kind of training they would be interested in, 34% (n=34/101) stated that they would like to get training in other hospitals providing gene therapies. In addition, 36% (n=36/101) of the respondents chose webinars and 37% (n=37/101) e-learning; 22% (n=22/101) selected symposium as a training model of interest (Figure 37).

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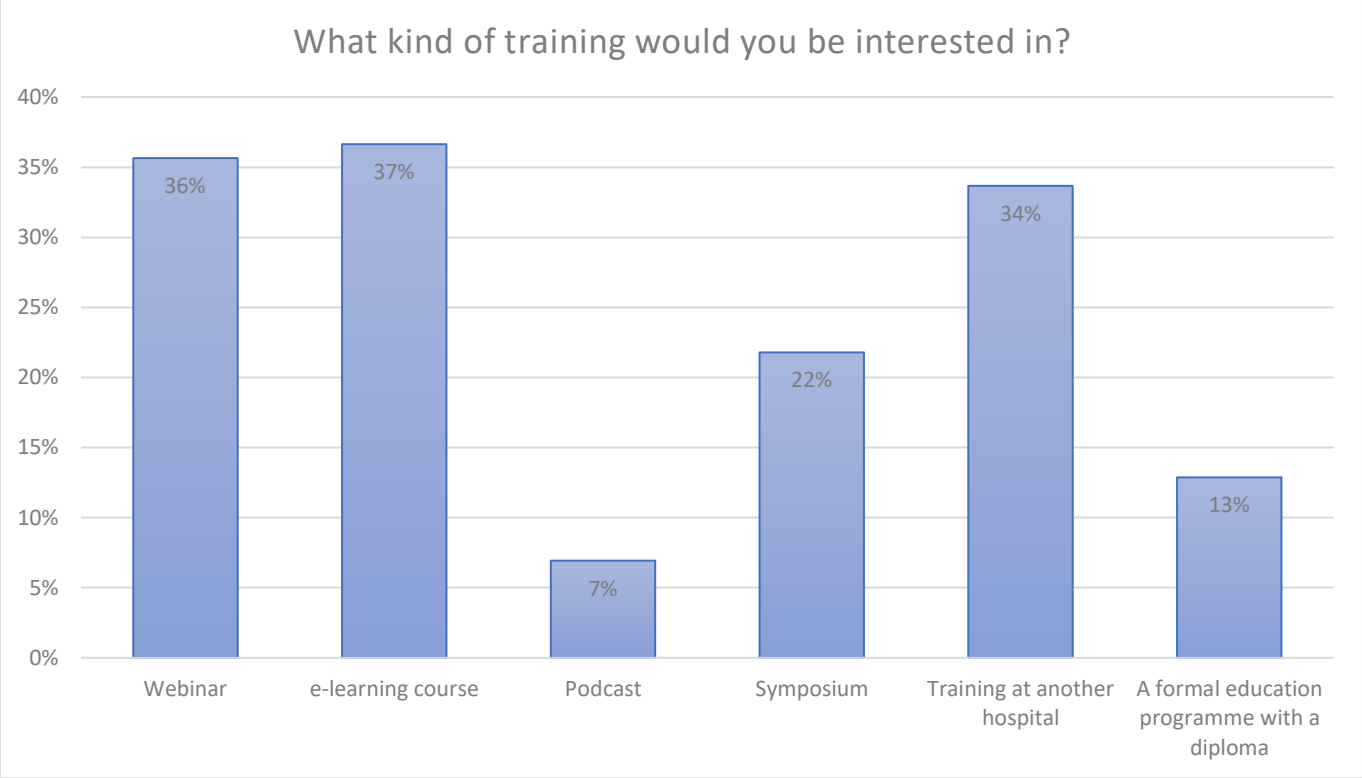

Figure 37: Percentage of responses (n=101) for the question 43 "What kind of training would you be interested in?

The questionnaire also asked if the hospital had a pharmacy coordinator who would be responsible for the interdisciplinary hospital team working with in-vivo gene therapies once these therapies got introduced there: 37% (n=19/49) answered yes while 45% (n=19/49) answered no, 19% (n=9/49) of the respondents didn't know.

Conclusions

This European survey of 216 hospital pharmacies is the largest survey on this topic undertaken to the knowledge of the SIG. The SIG survey found a wide range of preparedness for GTMP throughout Europe. Some of the Survey respondents mentioned that their hospitals are not yet prepared to use gene therapy medicines in the near future. Meanwhile other hospitals are quite advanced with national guidance on handling and administration of these medicines in place and many hospitals pharmacist already prepared, and staff trained. The United Kingdom is the most advanced in providing the relevant resources to hospital pharmacists on handling gene therapy medicinal products, following on from the development and resourcing of Advanced Therapy Treatment Centers (ATTCs)[12]. A pan UK pharmacy working group has published hospital pharmacy guidance for these products which is available on the Specialist Pharmacy Services (SPS) website.[13]

Handling GTMP is different from other medicines, however there is still a lack of guidance from the majority of the countries in Europe and many professional associations regarding their pharmaceutical application and handling. In

[12] https://www.theattcnetwork.co.uk/
[13] https://www.sps.nhs.uk

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2007, the EAHP guidelines were developed by gene medicine specialists' pharmacists to provide the minimum requirements for storage, transportation, preparation and dispensing, administration, disposal, decontamination, spillage, and accidental procedures based on the available evidence and practical experience. The SIG group considers that this document has been useful to help hospital pharmacists establish the infrastructures and developing adequate policies and protocols in their hospitals.

Therefore, the SIG agreed to review and update this document. Grants have been obtained from three pharmaceutical companies to fund a medical writer's time to undertake this work with the support of some of the SIG participants. An updated version will be launched in Fall 2023.

The survey also highlighted that there is an educational need for European hospital pharmacists on all aspects of handling GTMP. There are some hospital pharmacists across Europe with a lot of experience, but most of the hospital pharmacists require additional resources to enable them to safely deliver this new group of medicines in practice. We advocate for expanding training opportunities within the EAHP for the pharmacy workforce including webinars, training sessions in the annual conference and a masterclass. These educational programs could facilitate preparedness when approved gene therapy medicines become available more widely.

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Appendix I: SIG's membership

Name	Role	Country
Sylvain Auvity	Hospital Pharmacist & Associate Professor at the Necker Hospital-APHP and the University of Paris	France
Nanna Christiansen	Chief Children's Pharmacist and Associate Chief Pharmacist – Women's and Children's Services, Guy's and St Thomas' NHS Trust, London	United Kingdom
Joan Vinent Genestar (Chair of the SIG)	Lead Peadiatric Cancer Pharmacist at the Hospital Sant Joan de Déu in Barcelona	Spain
Liesbeth Huys	Pharmacist at the Ghent University Hospital	Belgium
Gráinne Johnston	Chief II Pharmacist – Aseptic Compounding Service Manager at the Mater Misericordiae University Hospital in Dublin	Ireland
Helle Bach Ølgaard McNulty	Head of Clinical Pharmaceutical Services department at the Capital Region Pharmacy	Denmark
Vera Pires	Hospital Pharmacist at the Portuguese Institute of Oncology in Lisbon	Portugal
Bertrand Pourroy	Pharmacy Hospital Practitionner at the Assistance Publique-Hopitaux in Marseille	France
Martin Schulz	Senior Medical Director Gene Therapy Platform, Global Medical Affairs, Rare Diseases at Pfizer	Germany
Nicola Stoner	Consultant Pharmacist – Cancer & ATMPs, Oxford Cancer and Haematology Centre, Churchill Hospital, Oxford University Hospitals NHS Foundation Trust	United Kingdom
Heidi Ekelund	Section leader at ATMP-center, Sahlgrenska University Hospital, Gothenburg, Sweden (M.SC Pharm, Hospital Pharmacy).	Sweden
Marcello Panni	Director UOC, Policlinico Universitario A. Gmelelli	Italy

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Appendix II: Training needs from the hospitals not providing gene therapy medicines.

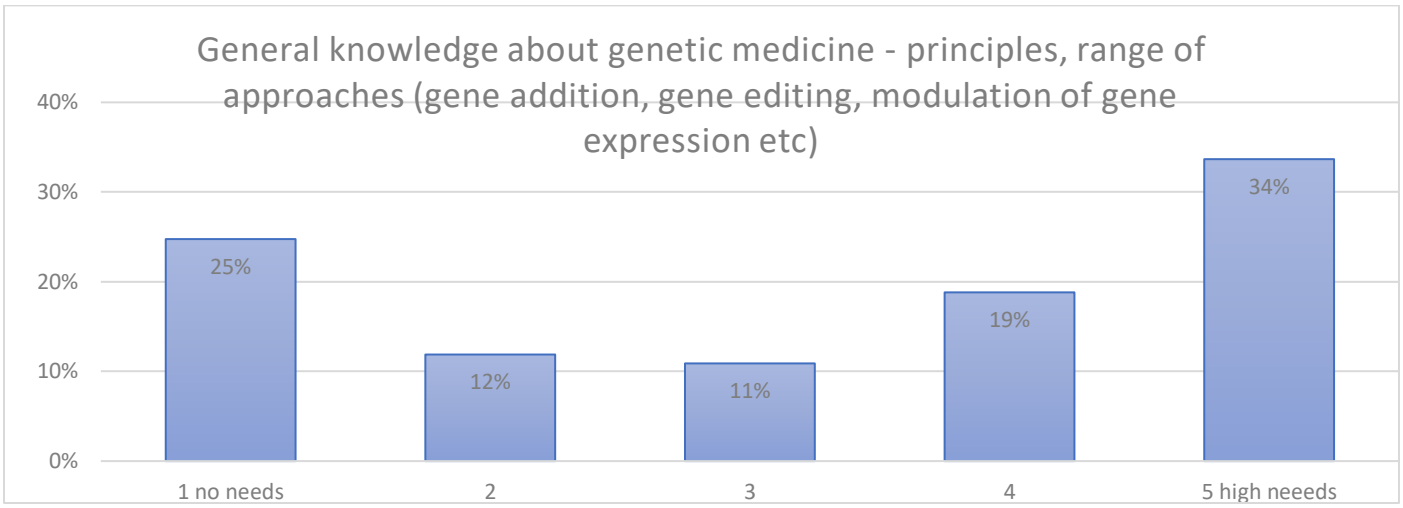

Figure 39: Percentage of responses (n=101) for the question 42.b) Rate your education and training needs..”

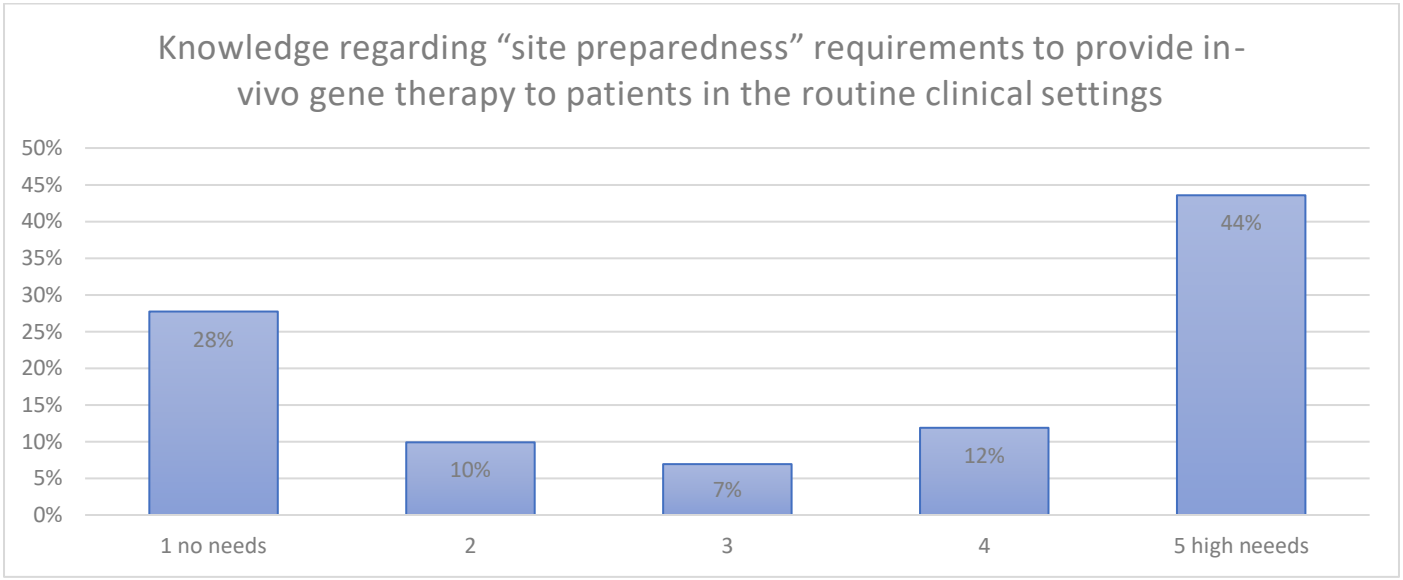

Figure 40: Percentage of responses (n=101) for the question 42.c) Rate your education and training needs..”

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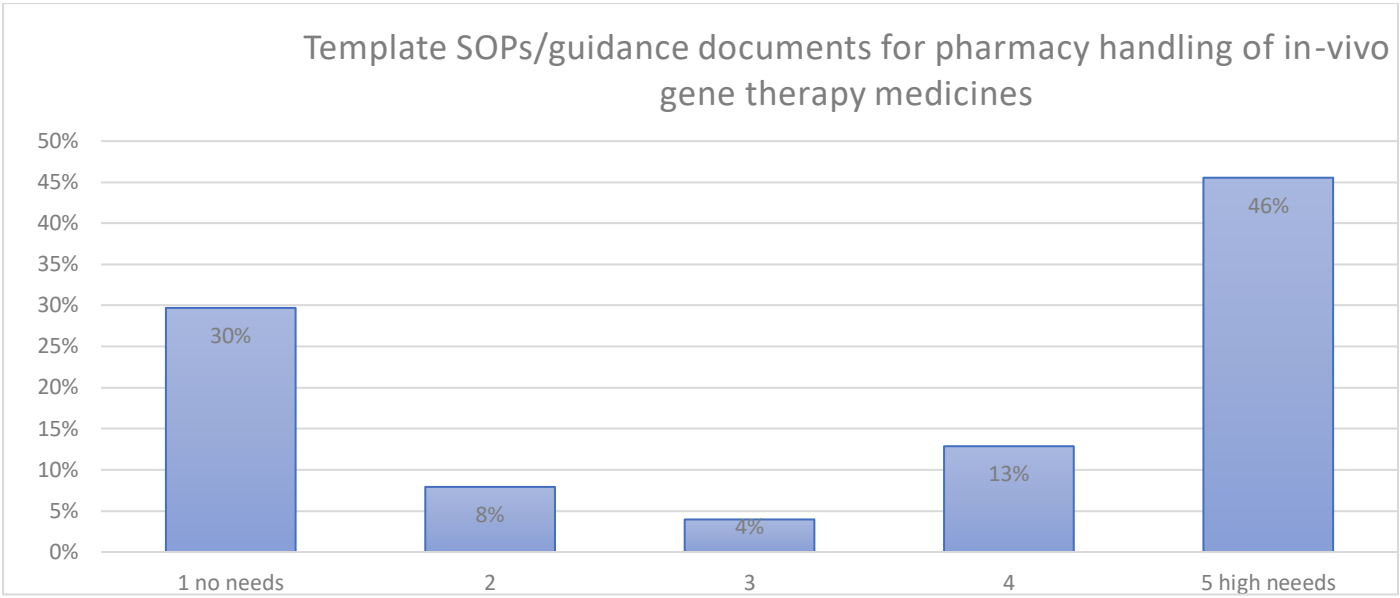

Figure 41: Percentage of responses (n=101) for the question 42.d) Rate your education and training needs."

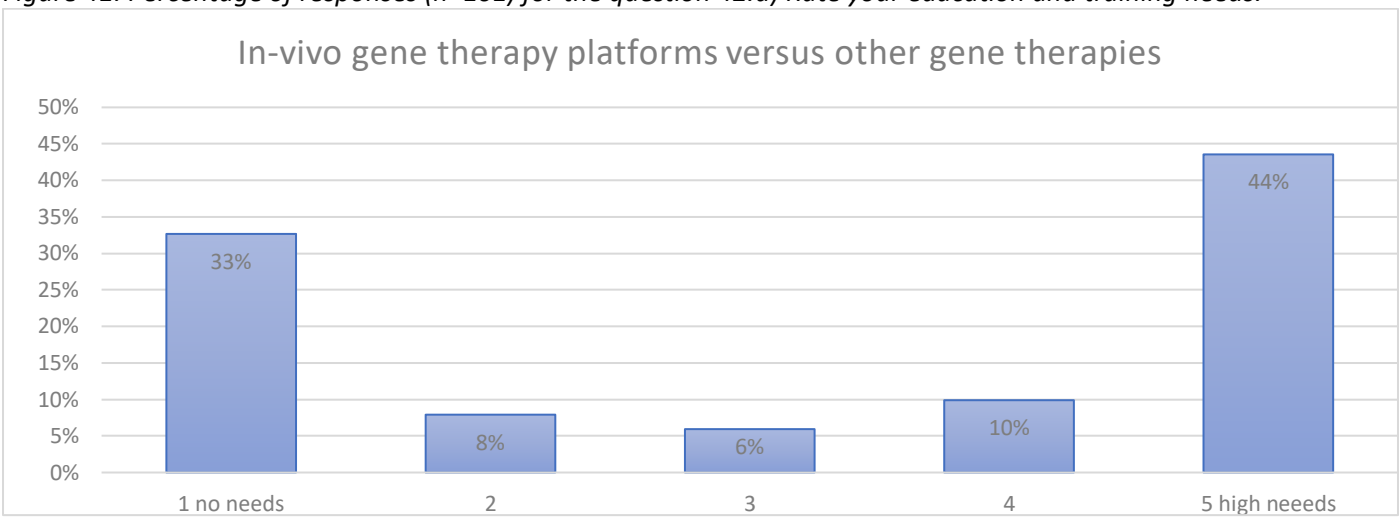

Figure 42: Percentage of responses (n=101) for the question 42.e) Rate your education and training needs."

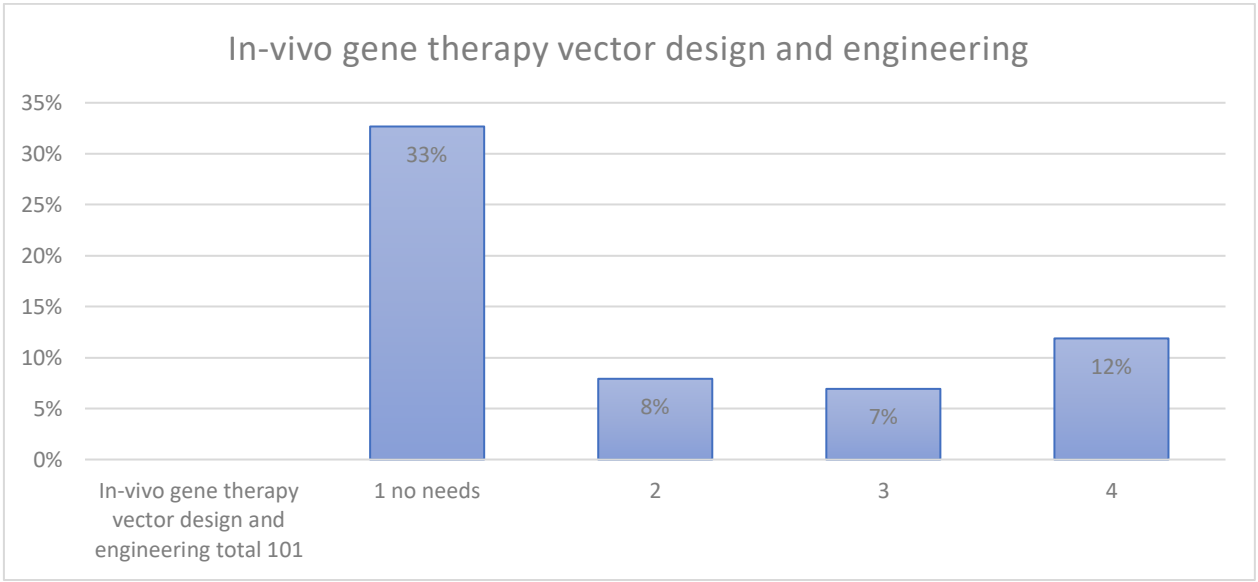

Figure 43: Percentage of responses (n=101) for the question 42.f) Rate your education and training needs."

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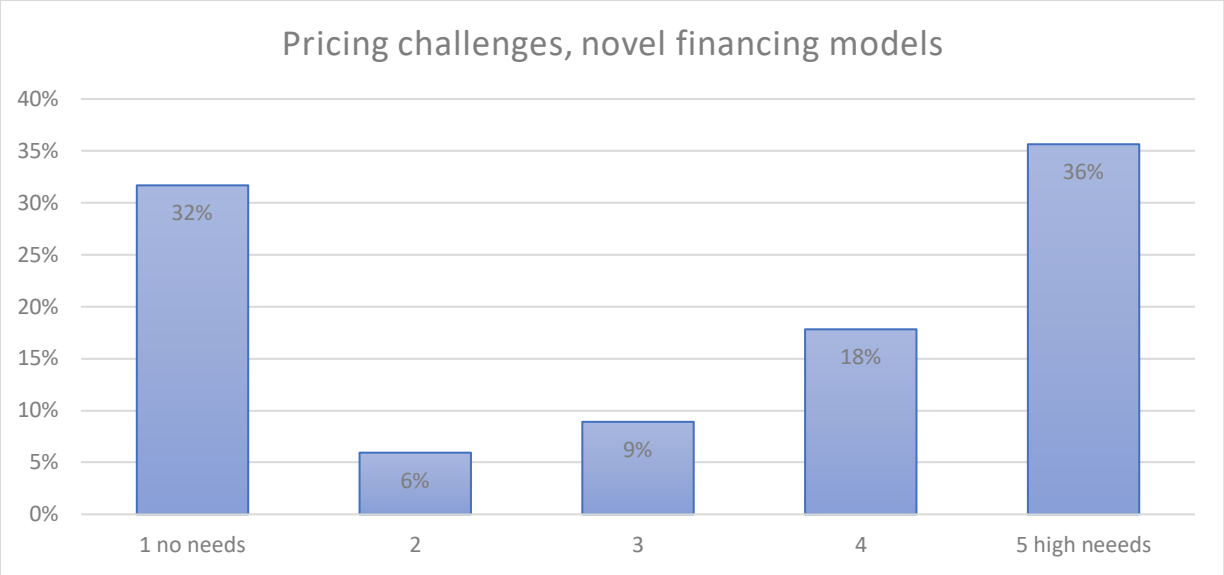

Figure 44: Percentage of responses (n=101) for the question 42.g) Rate your education and training needs."

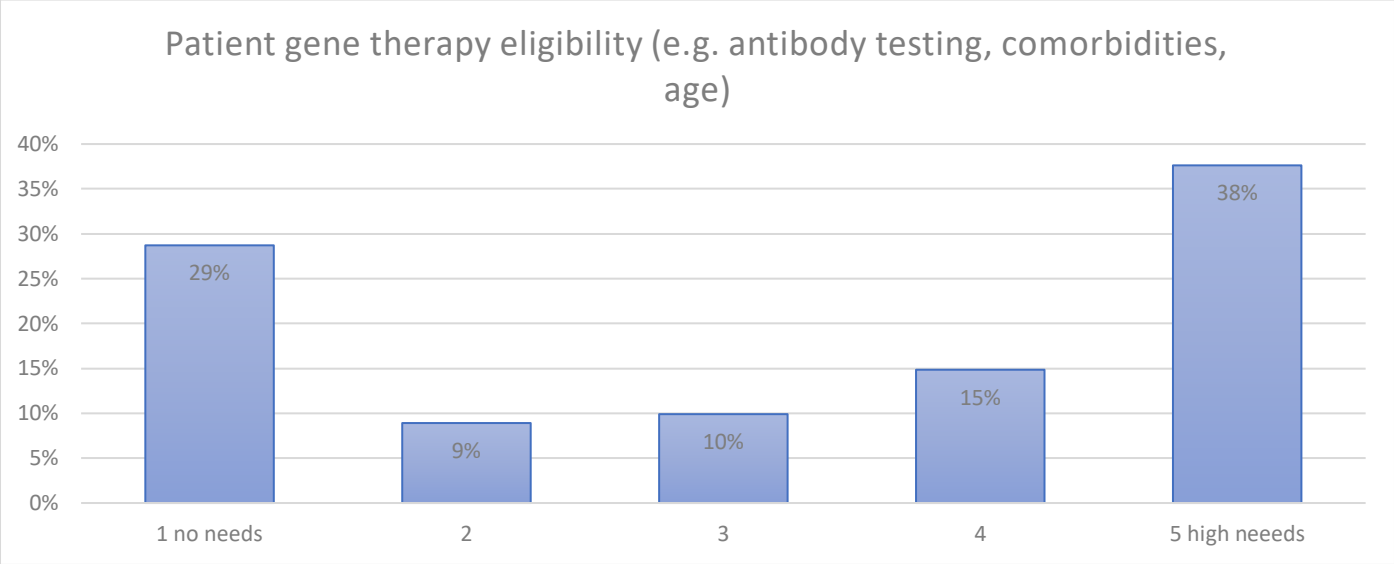

Figure 45: Percentage of responses (n=101) for the question 42.h)" Rate your education and training needs."

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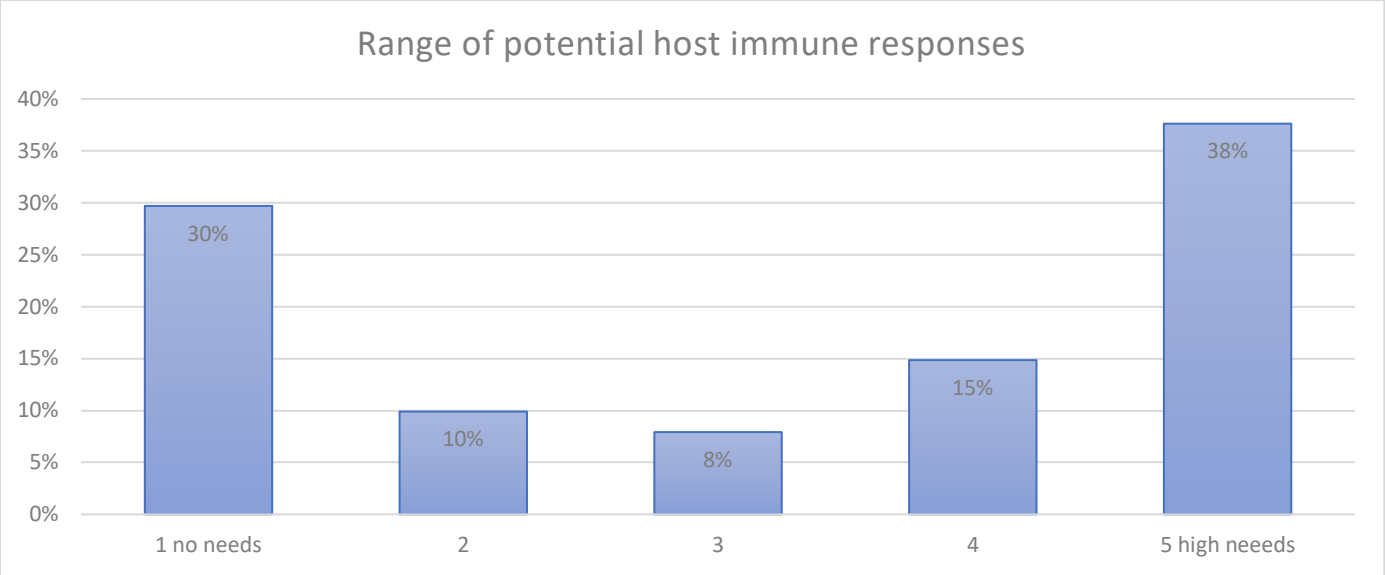

Figure 46: Percentage of responses (n=101) for the question 42.i)" Rate your education and training needs."

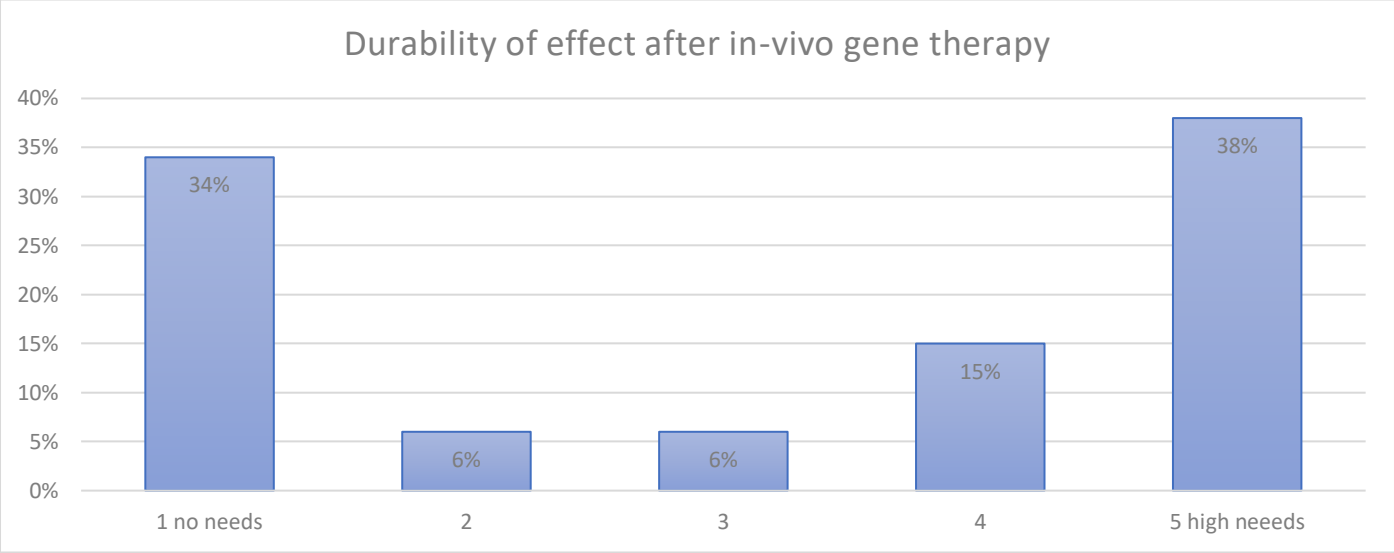

Figure 47: Percentage of responses (n=101) for the question 42.j)" Rate your education and training needs."

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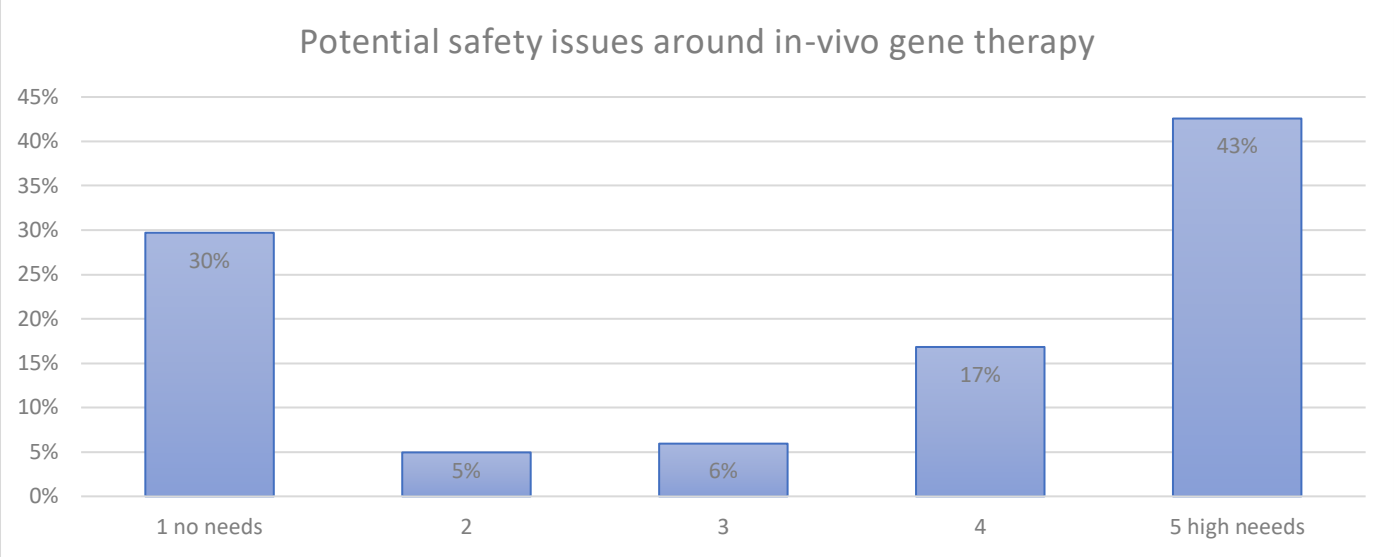

Figure 48: Percentage of responses (n=101) for the question 42.k) Rate your education and training needs."

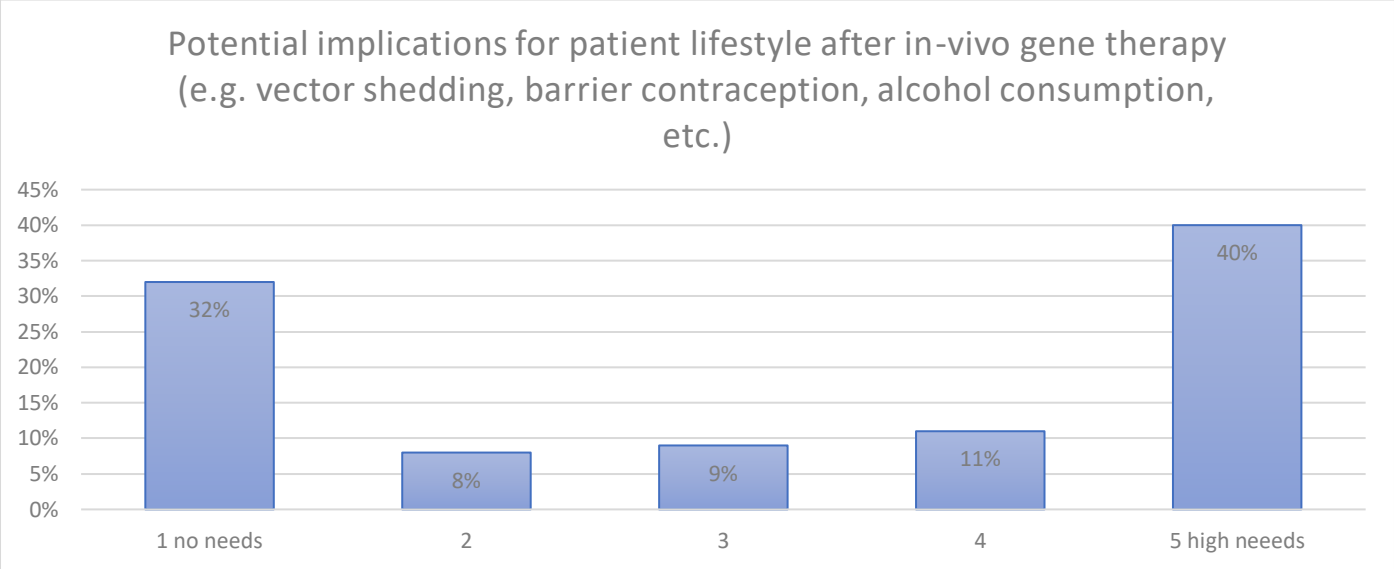

Figure 49: Percentage of responses (n=101) for the question 42.l) Rate your education and training needs."

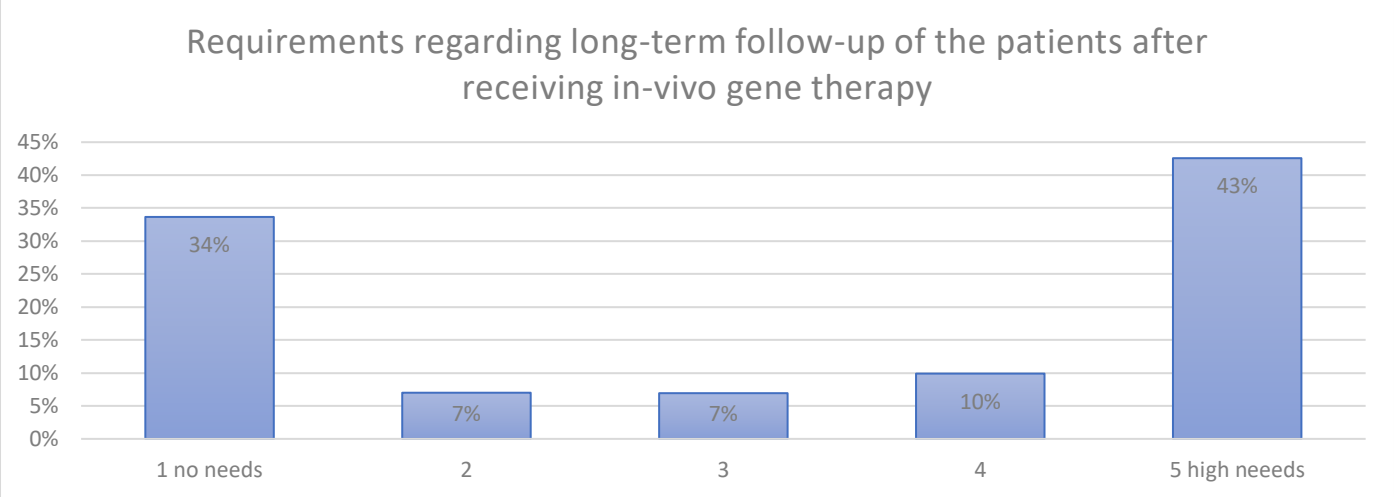

Figure 50: Percentage of responses (n=101) for the question 42.m) Rate your education and training needs."

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SPECIAL INTEREST GROUP | REPORT

Hospital pharmacist's preparedness for in-vivo gene therapy medicinal products

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