

Exploring Prospective Donors' Perspectives on Stem Cell Donation for Cell and Gene Therapy Research and Development

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Outline

- Overview of CGT & donations for stem cell research
 - The need for CGT
 - The challenges for CGT
 - Responses to the challenges for CGT
 - Overview of ethical concerns related to stem cell donation for research
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 - Qualitative data
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 - Limitations of findings
 - Implications for CGT community
 - Implications for future research

The need for CGT

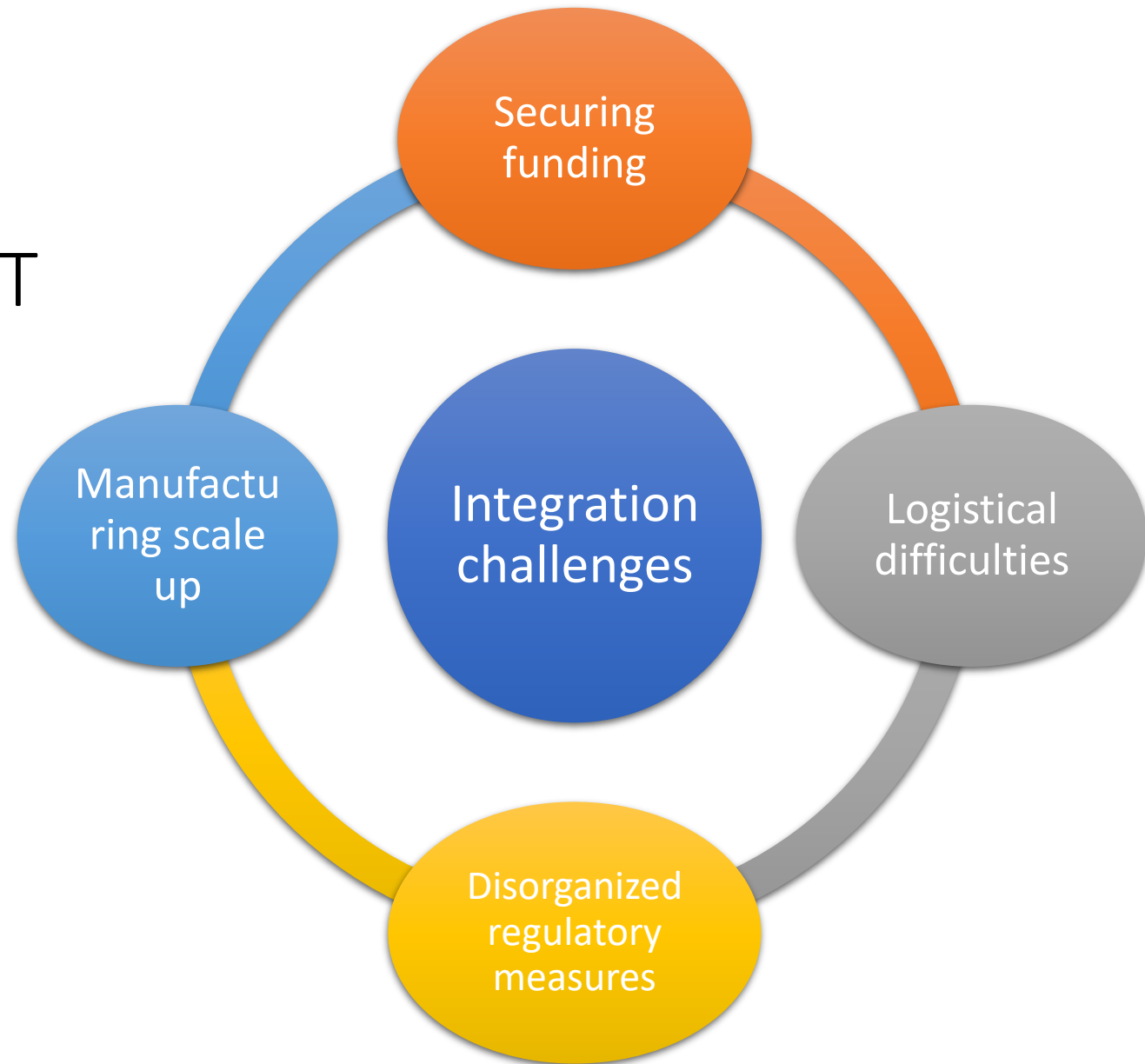
Validated stem cell therapies:

- Cornea
- Retina
- Bone marrow transplant

Under clinical or preclinical investigation:

- Cardiovascular
- Neurological
- Musculoskeletal

The challenges for CGT



Responses to the challenges for CGT

- The Cell Therapy Catapult

How we can help you



Industrialisation



Manufacturing



Regulatory



Health economics and market
access



Non-clinical safety

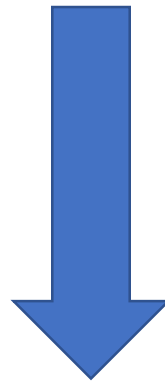


Clinical operations

Responses to the challenges for CGT

Situated at the cutting edge of translational research

Direct involvement with prospective donors to
provide stem cells



Ethical implications?

Overview of ethical concerns related to stem cell donation for research and biobanking

Ethical concern	Context under which ethical concerns emerge	Reference
Trust	<ul style="list-style-type: none"> • Among the public, trust in university-funded research on preserved stem cells in biorepositories is high but decreases when researchers associate with private, for-profit institutes. • Collaborations with private preservation enterprises may be viewed by the public as a compromise to public/academic institutes' commitment to their mission of public service and would lead to a loss of trust in public/academic sectors. 	Master, Z. et al.
Informed consent	<ul style="list-style-type: none"> • The issue of consent emerges in relation to subsequent commercialization of products and therapies that resulted from research on biobank samples. Consent as an ethical concern is also prominent when financial support by private bodies is provided to public biobanks. This is especially significant when this type of funding has not been attended to in the initial consent. 	Caulfield T. et al.
Privacy	<ul style="list-style-type: none"> • The potential for genetic health information leading to reidentification is associated with great concern for iPSC donors. This concern relates to the possible risk of discrimination and stigmatization such information may lead to. • Involvement of private funders may aggravate privacy concerns for biobank participants. Participants may deem their privacy violated if data sharing were to take place with for-profit organizations, inevitably compromising the public's trust in biobanks 	Isasi R. et al. Caulfield T. et al.
Commercialization	<ul style="list-style-type: none"> • Potential donors for iPSC research demonstrate concerns over the distribution of any resulting commercialized therapies. These concerns are demonstrated in the context of the immortalization of cell lines and the distribution of profit if therapies were to arise from them. 	Dasgupta et al.

It is essential to understand prospective donors' attitudes and perceptions on the sourcing of their stem cells by these charities to external organizations for the purpose of CGT developments.



Methods

- The survey was completed in 2019 by 2400 prospective stem cell donors enrolled in the Anthony Nolan stem cell registry
- Approximately 10,000 people on register were contacted with the aim for a 20% response rate

Main measures

Participants' level of understanding on the process of stem cell donation

Participants' level of understanding on work of the charity

Participants' willingness to donate cells to be used for research towards developing new therapies

Participants' degree of comfort with the charity collaborating with external organizations

Participants' degree of comfort with the charity receiving payment from external organizations

Data analysis

- Data entry and analysis of results were performed using SPSS software
- 94 participants provided qualitative responses which expanded upon and/or explained their quantitative responses in the survey

Results



QUANTITATIVE RESULTS



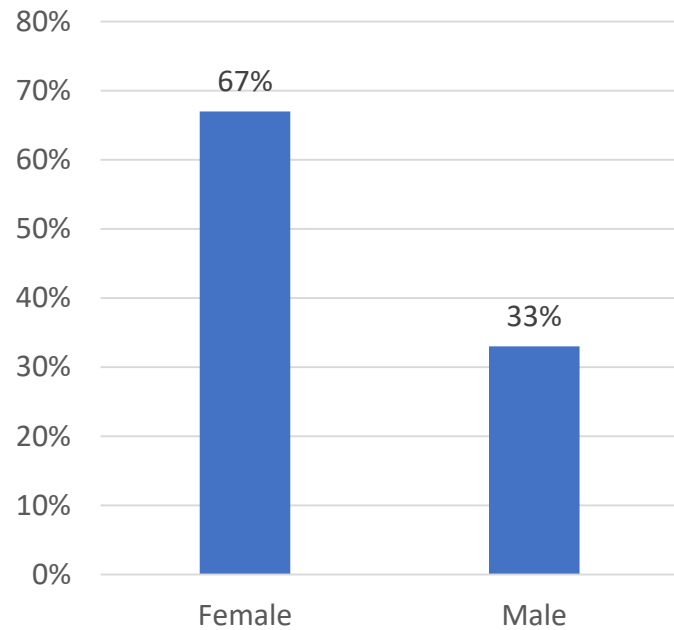
QUALITATIVE RESULTS

Quantitative Data

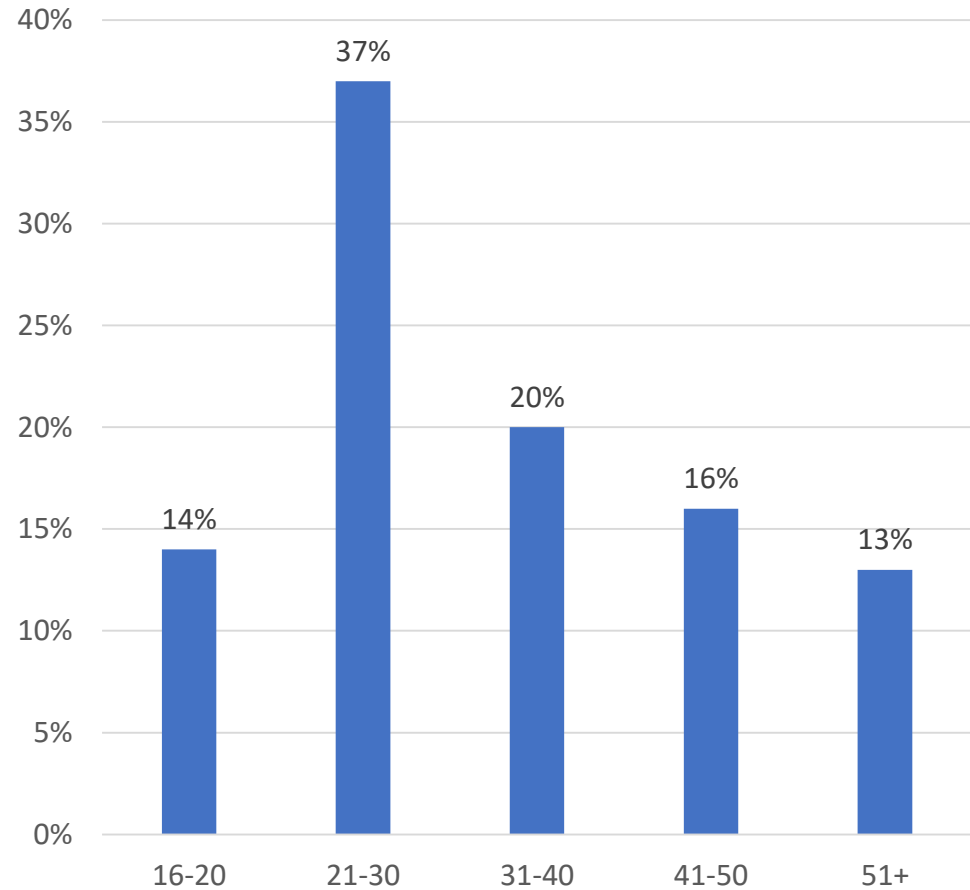


Demographics

Gender

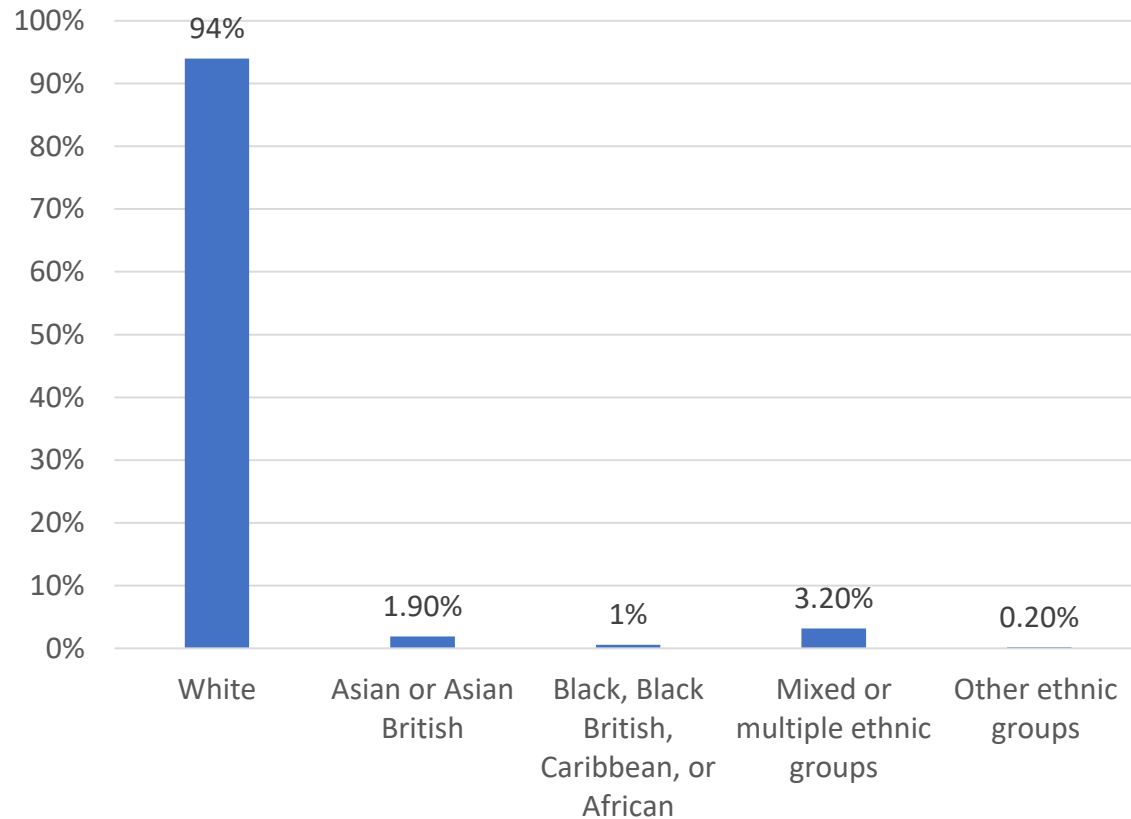


Age

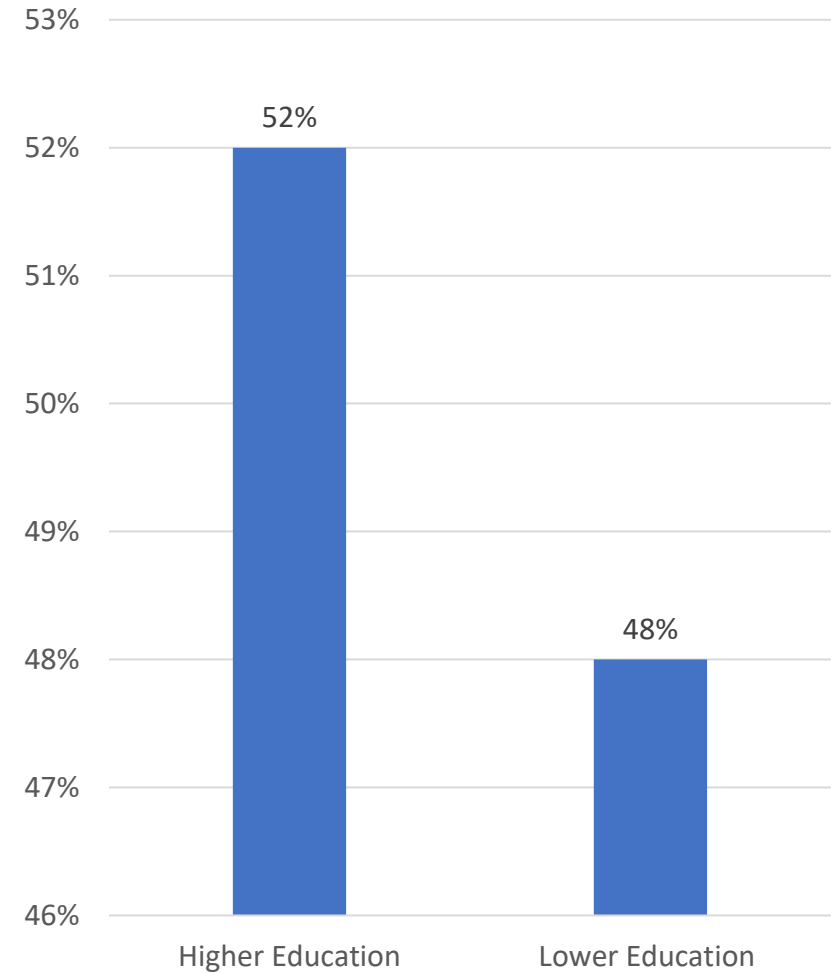


Demographics

Ethnicity



Education Level

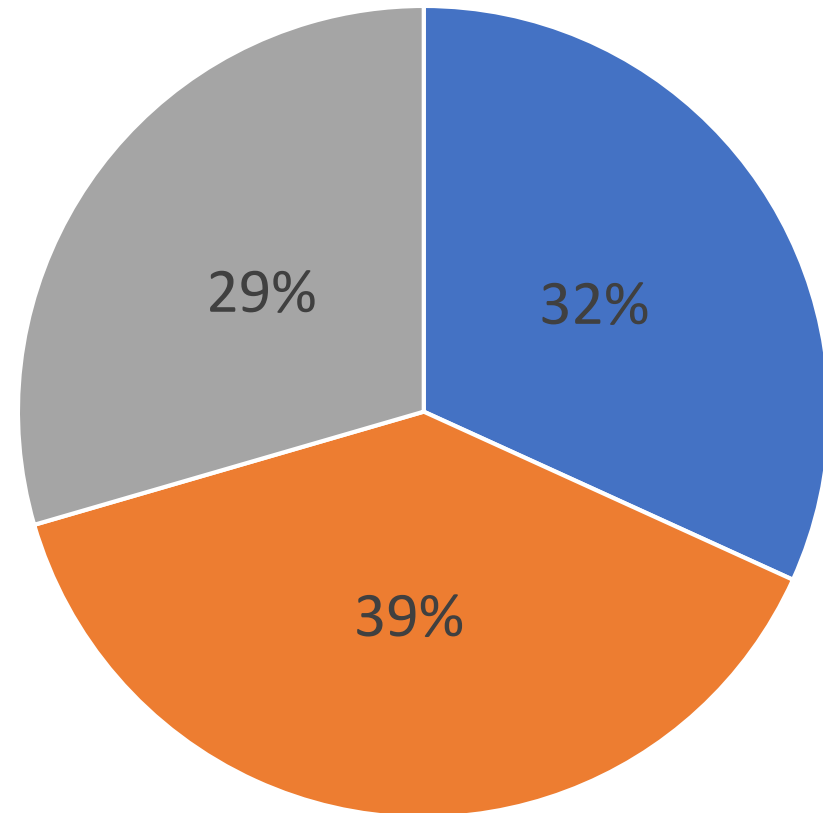


Understanding the process of peripheral blood stem cell collection

No statistically significant association between participants' level of understanding of PBSC and their willingness to donate for research (p value = 0.952)

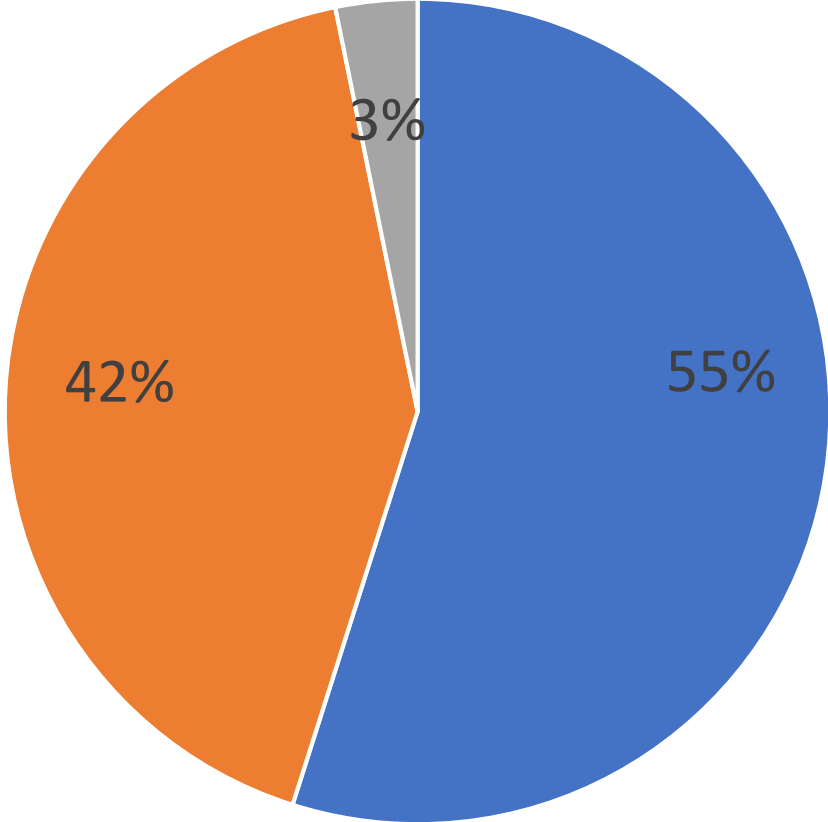
No statistically significant association between participants' level of understanding of PBSC and their degree of comfort with the charity collaborating with external organizations (p value = 0.764)

Understanding the process of stem cell donation via the bloodstream (PBSC)



- Good understanding - you would feel confident explaining this to another person
- Some understanding - you might struggle to explain this to another person
- Little/no understanding - you would need more information to explain to another person

Understanding the work of the charity



- Good understanding - you would feel confident explaining this to another person
- Some understanding - you might struggle to explain this to another person
- Little/no understanding - you would need more information to explain to another person

Understanding the work of the charity



No statistically significant association between participants' understanding of the work of the charity and their degree of comfort with external collaborations (p value = 0.327)

Statistically significant association between participants' understanding of the work of the charity and how comfortable they were with the charity receiving payments from external organizations (p value = 0.015)

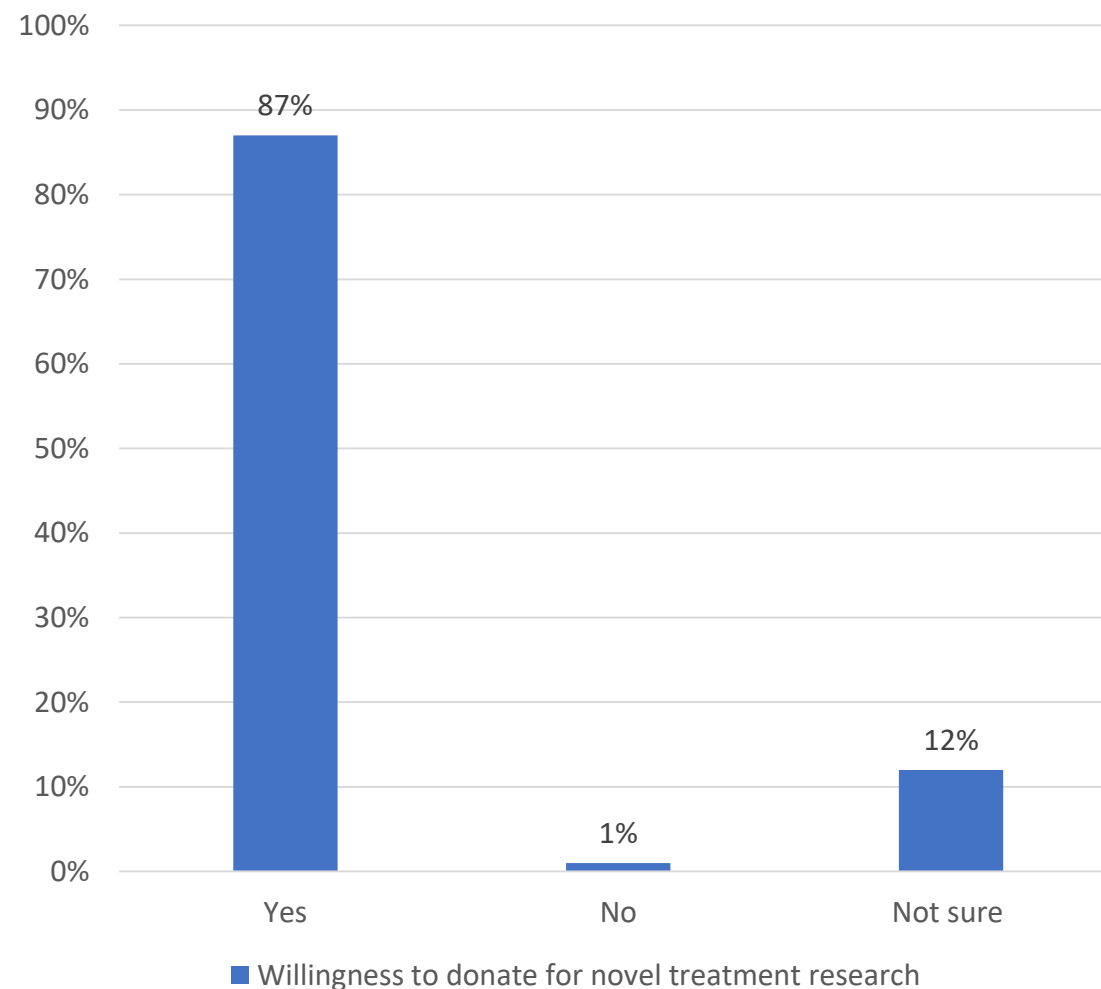
Good understanding of the work of the charity →
more agreement with regards to the charity
receiving payments (OR 2.893, 95%CI =
1.179,7.101)

Willingness to donate for novel treatment research

No statistically significant association between participants' level of education and their willingness to donate for CGT development (p value = 0.351)

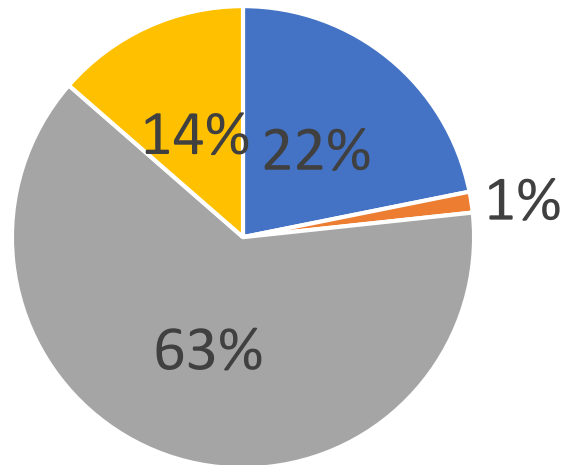
No statistically significant association between participants' ethnicity and their willingness to donate for CGT (p value = 0.194)

Willingness to donate for novel treatment research



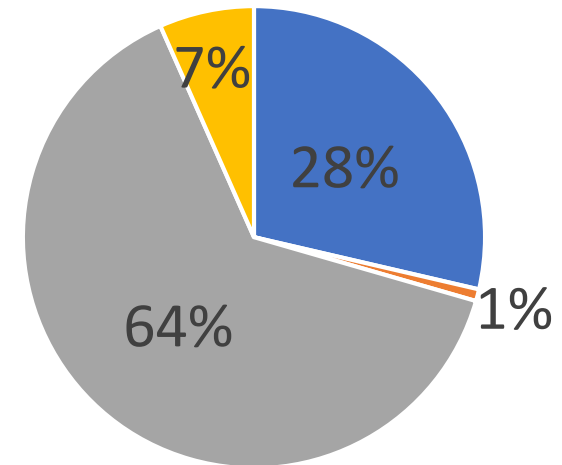
Willingness to donate for novel treatment research

Degree of comfort with the charity receiving payment among participants who are unsure of their willingness to donate for research



■ Strongly Agree ■ Strongly Disagree
■ Agree ■ Disagree

Degree of comfort with external collaborations among participants who are unsure of their willingness to donate for research



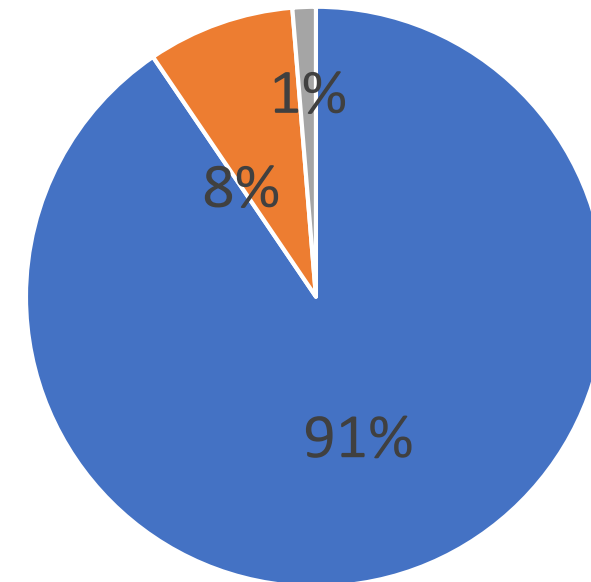
■ Strongly Agree ■ Strongly Disagree
■ Agree ■ Disagree

Degree of comfort with AN collaborating with external organizations

No statistically significant association between ethnicity and participants' degree of comfort with external collaborations (p value = 0.177)

Statistically significant association between participants' level of education with their degree of comfort with the charity collaborating with external organizations (p value = 0.005)

Degree of comfort with the charity collaborating with external organizations



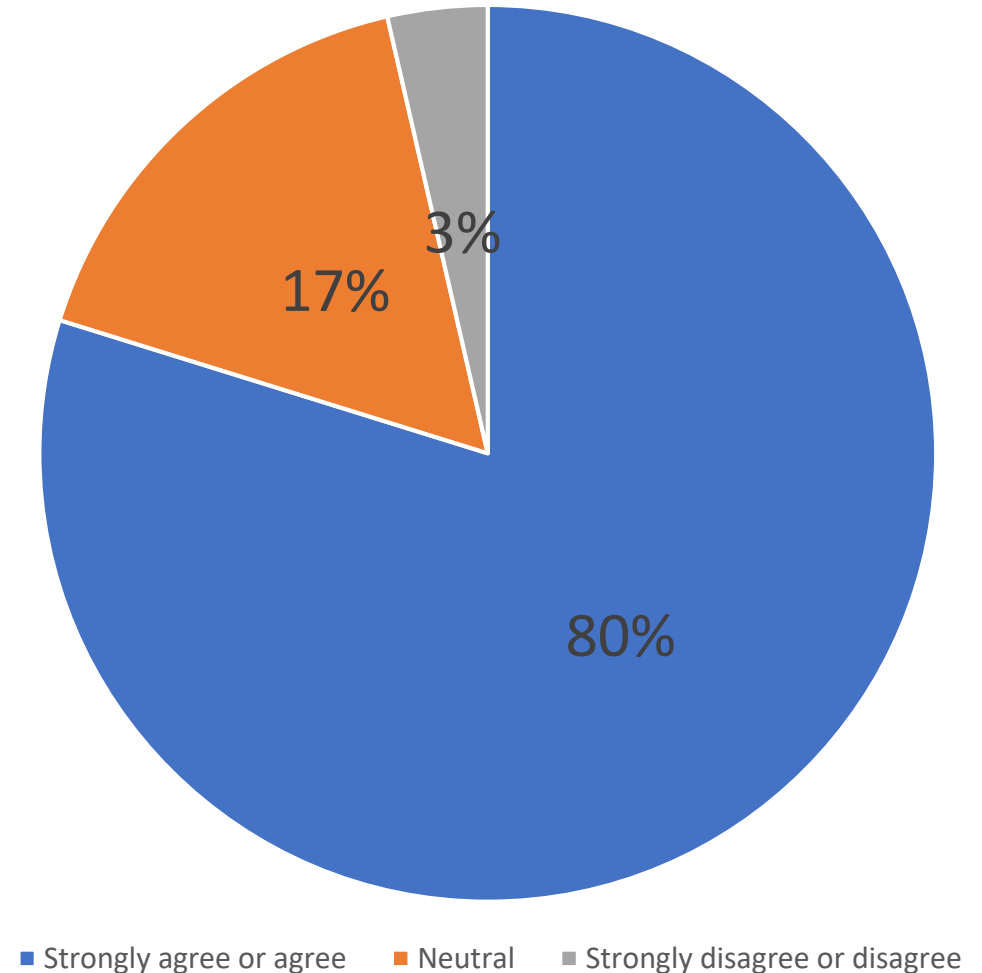
- Strongly agree or agree
- Neutral
- Strongly disagree or disagree

Higher level of education → less likely
to favour partnerships with external
organizations (OR = 0.312, 95%CI =
0.133,0.730)

Degree of comfort with AN receiving payment

- No statistically significant association between participants' ethnicity and their degree of comfort with the charity receiving payments from external organizations (p value = 0.214)
- Statistically significant association between participants' level of education and their degree of comfort with the charity receiving payments (p value < 0.001)

Degree of comfort with the charity receiving payment



Higher level of education → less likely
to favour receiving payment from
external organizations (OR = 0.40, 95%
CI = 0.247, 0.648)

Qualitative Data



Reasons underlying support for collaboration

Altruism

“I like to think that there is collaboration which is wider than the initial cause that the charity set up for. **To improve the health and quality of life** for current and future individuals, research and utilizing current resources such as the database of donors is a part of this. Just my opinion.” (Female, aged 40-51).

Hierarchy of donation

“I would be willing to allow some of a donation to be used if the majority of the donation was for a patient (i.e., **the research sample was a by-product**).” (Male, aged 40-51).

, “I would be happy to donate at an age where I would have to leave the register (so **my cells are no longer viable for treating someone but hopefully still viable for research**).” (Female, aged 21-30).

Concerns over collaboration

Privacy

“The only issue I would wish to be reassured on would be the **security of data sharing** between AN and other organisations and the **appropriateness of any payments between the parties**... this would extend to **DNA profiling, personal information security** etc.” (Male, aged 51+).

Commercialization and profiteering

“...I would object if the **pharmaceutical companies, then made millions** off those stem cells and **people in need had to pay a high price for their treatment.**” (Female, aged 51+).

“I feel comfortable about [charity] working with external organizations or receiving payment for them **only if this doesn't compromise in any way, directly or indirectly, the affordability of treatments for everyone.**” (Male, aged 21-30).

Facilitating stem cell donation

Transparency and informed consent

“I would feel more comfortable if **payments made to [charity] were publicized** and the **purpose of them were made clear.**” (Male, aged 21-30).

“[on collaboration with external organizations] this would be **greatly affected by who the agency/company/charity is...** this worry would be allayed if there were clauses within the contract which allowed the use of donations solely if the drug/therapy developed from those was provided at a **reasonable markup from cost.**” (Male, aged 31-40).

“I would be more cautious without significant safeguards, about [the charity] working or receiving money from pharmaceutical companies, **compared to, say, other charities.**” (Male, aged 51+).

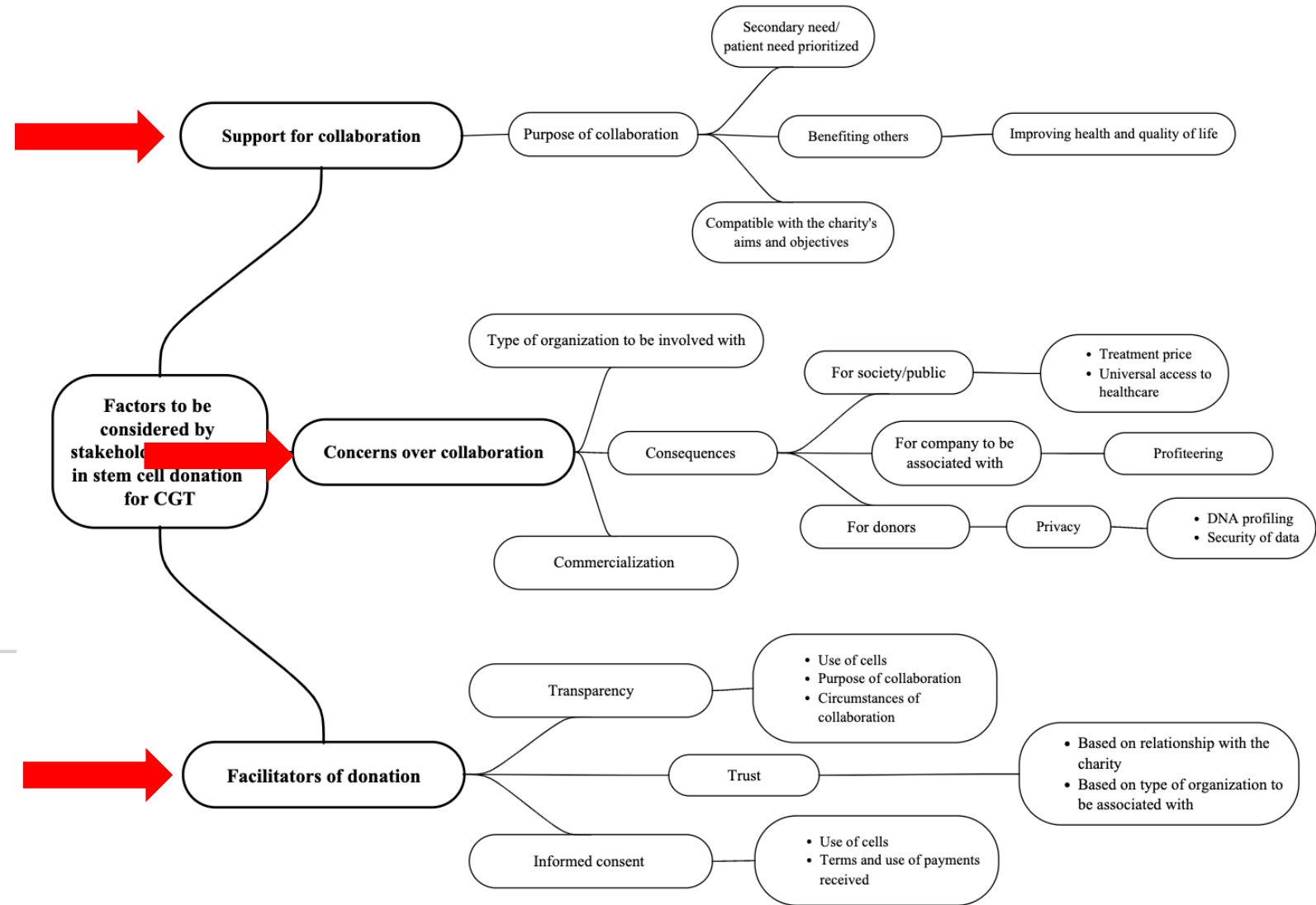
Facilitating stem cell donation

Trust

“I would be happy for [the charity] to work with other organisations/be paid by them as long as it was for reasons that were compatible with what [the charity] stands for...I trust [the charity] to make ethical and fair choices in who they work with.” (Female, aged 21-30).

“I understand that provision of donations to third parties in exchange for money is a necessary evil...I hope some of your affiliates are also charitable organisations.” (Female, aged 20-31).

Overview of factors to be considered by stakeholders engaged in stem cell donation for CGT



Limitations of findings

Role of pharmaceutical companies in relation to the COVID-19 pandemic

- 24% increase in the public's positive views of the pharmaceutical industry since the pandemic

Sample population

- Majority of participants were white (94%)
- Minority groups scarcely represented

Implications for CGT community

Access to stem cells

- Therapeutic donations take precedence over donations for research purposes
- Constructed hierarchy of donation → limitation on access to SC for CGT research
- Recent data: age does not limit iPSC application for regenerative therapies
- Target older prospective donors

Implications for CGT community

Privacy and security of genetic material

- Results of the survey support the literature
- To facilitate SC donations & maintain trust → General Data Protection Regulation (GDPR)

Transparency & informed consent

- Higher education → less likely to favour external collaborations & funding
- Transparent consent process
- Full disclosure: nature of partnership, payment process, potential commercial value

Implications for CGT community

Donors' moral responsibility

- Donors' perceptions and understanding of the process of CGT development and manufacturing

Hesitancy to donate stem cells for novel treatment research

- Inquiry into factors influencing donors' decisions to donate for CGT research

Proactive educational initiatives

- Increasing donors' awareness on the work of the charity

Implications for future research



Insight into donors' perceptions on pharmaceutical companies vs. charitable organizations



Donors display moral obligation & keenness to influence the direction of any donations made



What is deemed to be 'ethical' engagement between charitable and commercial organizations?



How can trust be constructed and when is it undermined?

Thank you!