

anos (DP = 15,9). A prevalência de tabagismo, elitismo e obesidade foi 13,8%, 12,5% e 24,6%, respectivamente. A prevalência de eventos trombóticos foi 9,6% (n = 17). Entre os fatores associados ao evento trombótico, pacientes com histórico de trombose venosa profunda apresentaram percentual superior para evento trombótico quando comparados com aqueles sem histórico (50 vs 8,2%; $p < 0,001$ – RR = 2.14). Das características clínicas, pacientes com tumores de grau III e IV, e IDH não mutante tiveram maiores percentuais para eventos trombóticos, porém sem associação significativa ($p > 0,050$). Ao analisar a terapêutica pós-cirúrgica, pacientes que realizaram radioterapia e quimioterapia apresentaram RR = 1,14 (IC95% 1,07 – 1,21) e RR = 1,11 (IC95% 1,02 – 1,20), respectivamente. Quanto ao óbito dos pacientes, 17,9% apresentaram evento trombótico enquanto entre os vivos, 8,5% apresentaram o evento ($p = 0,247$ – RR = 1,11; IC95% 0,93 – 1,33). O tempo de seguimento mediano entre o evento trombótico e o status final foi de 2,8 meses (1 dia a 9 meses) e 3,84 meses (1 dia a 12 meses), respectivamente, para os pacientes que vieram a óbito e vivos ($p > 0,050$). **Discussão:** Nesta amostra preliminar, o estudo indica que pacientes com evento trombótico apresentam maior risco de evoluir a óbito em, aproximadamente, 3 meses pós-evento. Quanto ao histórico de saúde, a presença de trombose venosa profunda prévia e as terapêuticas pós-cirúrgicas associaram-se ao evento trombótico pós-diagnóstico. Embora a associação entre o evento trombótico e a demais características clínicas (i.e., grau tumoral, IDH, dentre outras) não tenha sido significativa, tais achados podem ser atribuídos ao tamanho amostral. **Conclusão:** Em pacientes com neoplasia maligna do sistema nervoso central, os fatores associados ao evento trombótico foram históricos de trombose venosa profunda, realização de radioterapia e quimioterapia.

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SOCIAL RETURN OF INVESTMENT ANALYSIS OF GENE THERAPY FOR HEMOPHILIA IN BRAZIL: STUDY PROTOCOL

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Objectives: To estimate the social return of investment (SROI) of gene therapy (Tx) for hemophilia A and B in Brazil, and to discuss a framework for measuring social and long-term value of advanced therapy medicinal products (ATMPs). **Methods:** SROI forecast analysis. A conceptual logical model was developed as a first step by a multi-disciplinary team to map (i) main stakeholders; (ii) inputs and outputs expected along the implementation of gene Tx for hemophilia in the Brazilian public health system; and (iii) short-, medium-, and long-term outcomes. This conceptualization is the foundation of the Theory of Change that will underpin the SROI analysis. Patients, family members, and health care professionals (HCPs) were mapped as main stakeholders. Study protocol will be submitted to an Institutional Research Board and

participation will be voluntary and conditioned to informed consent. Patients and family members will be enrolled from patients' associations. One-to-one semi-structured interviews will be carried out using an online platform. Forty stakeholder interviews are planned, with participation of at least 20 patients. Family members and HCPs will account for the other 20 interviews. Theoretical saturation will be assessed through a saturation table and a pre-defined code-book. Questions will address the impact of disease and its treatment on the ability of performing daily life activities and social interactions. The impact of gene Tx on the number of bleedings will be derived from pivotal randomized controlled trials and extrapolated over time. Reduction of health care expenditures related to prophylaxis and complication management will be estimated, as well as the reduction of social security expenditures. Financial proxies will be applied for valuing intangible outcomes. Social value will be calculated by the amount of tangible and intangible earnings multiplied by the respective financial values, with adjustments for attribution, deadweight, and drop off. SROI per dollar invested will be presented as the main study outcome. **Results:** Results are expected to be obtained and presented in scientific Journals by the end of 2024. **Discussion:** There are challenges for valuing ATMPs, including lack of definition and valuation of cure for chronic diseases, approach for qualifying cost-savings compared with current treatments, and choice of perspective. Some frameworks have been developed to address limitations of traditional health technology assessments (HTA) methods, considering the specificities of ATMPs and the limitations of using quality-adjusted life years (QALYs) in some situations. The ISPOR Value Flower accounts for novel value elements, such as severity of illness, value of insurance, value of hope. The generalized risk-adjusted cost-effectiveness (GRACE) approach was developed to account for the adjustment of a willingness-to-pay threshold to untreated illness severity or pre-existing permanent disability, and the differentiation between life expectancy and quality of life across health states. SROI is a comprehensive, well-described method of cost-benefit analysis that considers tangible and intangible outcomes, allowing for the measurement of long-term value from the societal perspective. **Conclusion:** This study will provide evidence on the social value of gene Tx for hemophilia, advancing the methods for valuing ATMPs. Results may support future HTA, along with cost-effectiveness analysis and budget impact analysis.

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WHAT ARE THE PERCEPTIONS AND MOST RELEVANT TREATMENT OUTCOMES REGARDING GENE THERAPY ACCORDING TO PEOPLE WITH HEMOPHILIAS IN BRAZIL? STUDY PROTOCOL FOR A QUALITATIVE PATIENT PREFERENCE STUDY

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