**Table S1**. Estimated timing from Phase I, II and III to launch for three created scenarios.

|  |  |  |  |
| --- | --- | --- | --- |
| Development stage | Optimistic launch | Base launch | Pessimistic launch |
| Phase I | 8 years | 9 years | 10 years |
| Phase II | 6 years | 7 years | 8 years |
| Phase III | 4 years | 5 years | 6 years |

**Table S2**. Overview of clinical trials for CGTs across different diseases.

|  |  |  |  |  |
| --- | --- | --- | --- | --- |
| Disease group | Phase I | Phase II | Phase III | Total |
| Cardiovascular | 7 | 16 | 4 | 27 |
| Dermatology | 0 | 2 | 2 | 4 |
| Haematology (non-oncology) | 13 | 30 | 8 | 51 |
| Haemato-Oncology | 103 | 51 | 6 | 160 |
| Immunology | 6 | 24 | 0 | 30 |
| Metabolic | 23 | 69 | 9 | 101 |
| Neurology | 30 | 40 | 10 | 80 |
| Solid tumour | 108 | 71 | 1 | 180 |
| Ophthalmology | 14 | 48 | 14 | 76 |
| Total | 304 | 351 | 54 |  |

Note: Data extraction covered all the trial information available as of January 2023

**Table S3**. List of forecasted CGT’s target indication in France, 2023-2030.

|  |  |
| --- | --- |
| Forecasted year of launch | Forecasted CGTs indication |
| 2023 | * Haemophilia B * Sickle Cell Disease * Mantle cell lymphoma and acute lymphoblastic leukemia |
| 2024 | * Choroideremia * Aromatic L-amino Acid Decarboxylase (AADC) Deficiency * Recurrent Glioblastoma Multiforme * Limb Girdle Muscular Dystrophies (LGMD) * Amaurosis |
| 2025 | * Homozygous Familial Hypercholesterolemia (HoFH) * Variant Late-Infantile Neuronal Ceroid Lipofuscinosis * Peripheral Arterial Disease * Spinal Muscular Atrophy * X-Linked Chronic Granulomatous Disease |
| 2026 | * Ornithine Transcarbamylase (OTC) Deficiency * Leber's hereditary optic neuropathy * Epidermolysis Bullosa * Hodgkin Lymphoma * Achromatopsia * Severe Combined Immunodeficiency, X-Linked * Hemophilia B |
| 2027 | * Lymphoma * Neoplasms * Glioblastoma Multiforme * Pancreatic Cancer * ADA-SCID * Lysosomal Storage Disease * Duchenne Muscular Dystrophy * B-Cell Non Hodkin Lymphoma |
| 2028 | * X-Linked Retinitis Pigmentosa * Multiple Myeloma * Hemophilia A * HIV-1-related high-risk lymphoma * Fabry Disease * Non Small Cell Lung Cancer * Acute Lymphoblastic Leukemia |
| 2029 | * Dry Age-related Macular Degeneration * Sickle Cell Disease * Chronic Lymphocytic Leukemia * Acute Myeloid Leukemia * Lymphocytes * Hepatocellular Carcinoma * Stage IV Cutaneous Melanoma * Melanoma |
| 2030 | * Acute Lymphoblastic Leukemia * Wilson Disease |