

CELL- PID FINAL REPORT

Section: Description of the main S & T results/foregrounds

WP4 – MANUFACTURING OF VECTORS AND CELLS

Figure 1 - CliniMACS Prodigy®

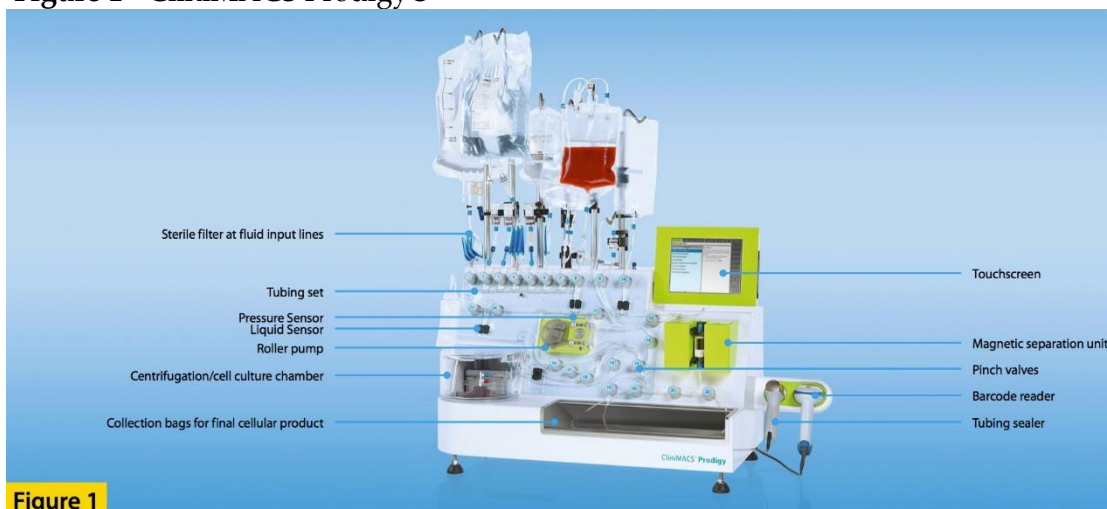


Figure 1

Figure 2 – The prototype process

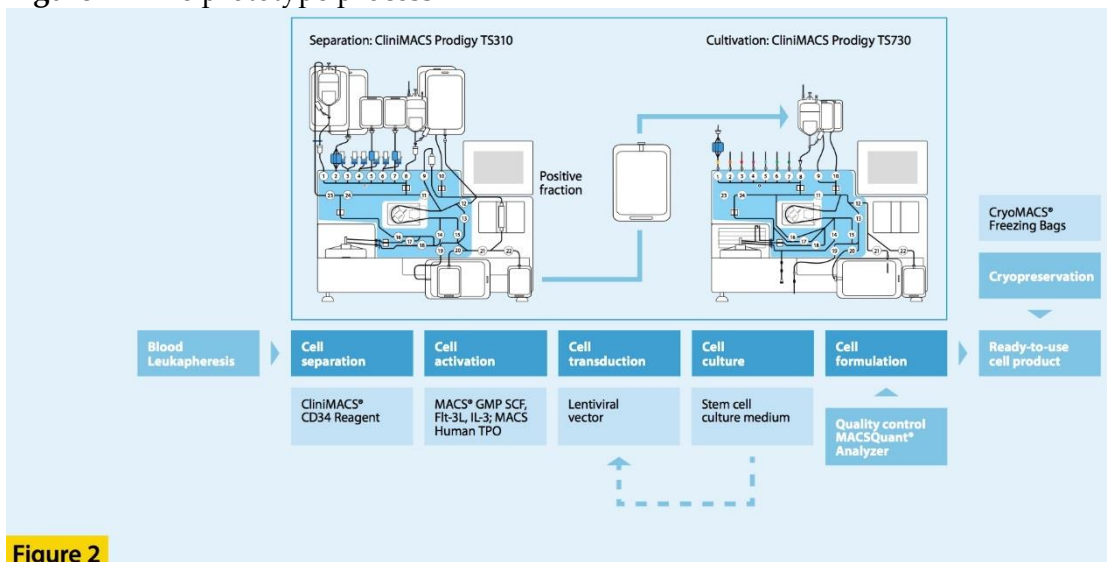
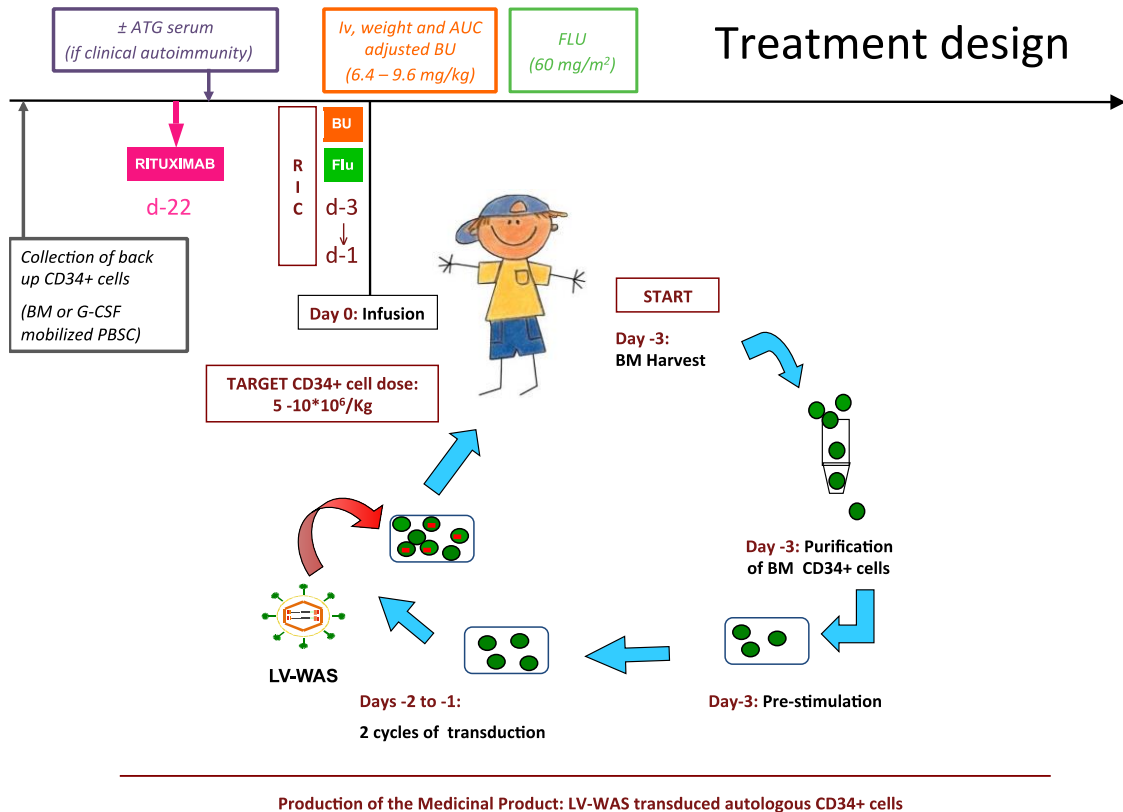


Figure 2

WP10 –PHASE I/II TRIAL OF SIN LENTIVIRAL GENE THERAPY FOR WISKOTT-ALDRICH SYNDROME

Figure 3 – Production of the Medicinal Product: LV-WAS transduced autologous CD34+cells





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In the section: description of the potential impact (including the socio-economic impact and the wider societal implications of the project

Table 1. Ongoing clinical trials on primary immunodeficiencies in the world and related publications. Several CELL PID partners are leading these activities.

TABLE 1. ONGOING GENE THERAPY CLINICAL TRIALS FOR PRIMARY IMMUNODEFICIENCY

Disease	Country	Sponsor	Vector	Outcome	Reference	Clinical Trials.gov ref no.
SCID-X1	France	Assistance Publique—Hôpitaux de Paris, Great Ormond Street Hospital, Children's Hospital Boston	SIN γ -RV	8/9 patients alive, immune recovery with clinical benefit	Haccin-Bey-Abina et al. ¹⁹	NCT01410019
	UK					NCT01175239
SCID-X1	US	St. Jude Children's Research Hospital, National Institute of Allergy and Infectious Diseases	SIN-LV	Two young adult patients, improved IgG production	De Ravin et al. ³⁰	NCT01129544
						NCT01512888
ADA-SCID	Italy	GlaxoSmithKline	γ -RV	15/18 patients off ERT, clinical benefit	Aiuti et al., ⁵⁴ Cicalese et al. ¹¹⁷	NCT00598481
ADA- SCID	UK	Great Ormond Street Hospital	γ -RV	4/6 patients off ERT, clinical benefit	Gaspar et al. ⁵⁵	NCT01279720
ADA- SCID	US	Donald B. Kohn, UCLA	γ -RV	9/10 patients off ERT, clinical benefit	Candotti et al., ⁵⁶ Carbonaro Sarracino et al. ¹¹⁸	NCT00794508
ADA-SCID	UK	Great Ormond Street Hospital; Donald B. Kohn, UCLA	SIN-LV	5 patients, immune and metabolic recovery	Gaspar et al. ¹¹⁹	NCT01380990
	US					NCT01852071
WAS	Italy	IRCCS San Raffaele	SIN-LV	6 patients, immune, hematological and clinical improvement	Aiuti et al., ⁸⁷ Scaramuzza et al. ¹²⁰	NCT01515462
WAS	France	Genethon	SIN-LV	France: 4/5 patients alive, clinical improvement	Bosticardo et al. ⁸⁹	NCT01347346
WAS	UK					NCT01347242
WAS	US	Children's Hospital Boston	SIN-LV	2 patients, immune and hematological improvement	Williams ¹²¹	NCT01410825
CGD	Germany	Hubert Serve, Johann Wolfgang Goethe University Hospitals	SIN γ -RV			NCT01906541
CGD	Germany	Genethon	SIN-LV		Kaufmann et al. ¹⁰⁹	NCT01855685
	Switzerland					NCT02234934
	UK					
	US	UCLA				

ADA, adenosine deaminase; CGD, chronic granulomatous disease; ERT, enzyme replacement therapy; LV, lentiviral; RV, retroviral vector; SCID, severe combined immunodeficiency; SIN, self-inactivating; WAS, Wiskott-Aldrich syndrome.
 Studies are classified as active and ongoing based on information retrieved from ClinicalTrials.gov. Updated information on recruitment status can be found on ClinicalTrials.gov.