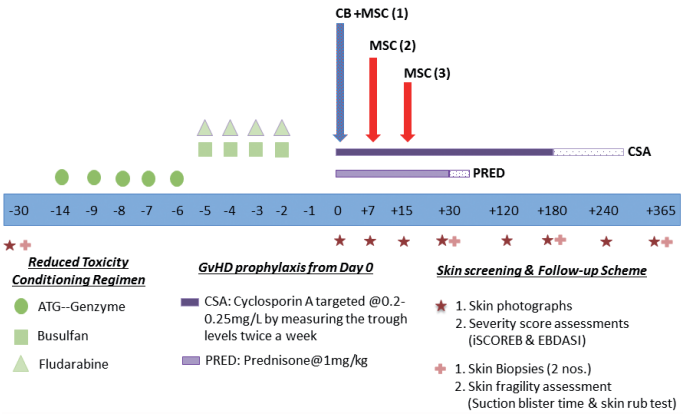

Fig. S1. Study protocol for allogeneic haematopoietic cell transplantation trial (CB+MSC4EB Trial) with myeloablative reduced toxicity conditioning, initially consisting of fludarabine i.v. (cumulative dose: 160 mg/m^2 in 4 days) and busulfan i.v. (cumulative target area-under-the-concentration-over-time curve (AUC): 90 (85–95) mg*h*L in 4 days) together with serotherapy (ATG-genzyme), later adjusted according to an individualized dosing regimen with therapeutic drug monitoring, as described previously (9, 10). This regimen was followed by an unrelated cord blood transplantation (CBT) (selection criteria: total nucleated cell (NC) count >3×10^7/kg for the 8–10/10 matched units and >5×10^7/kg for the 6–7/10 matched units)) and third-party mesenchymal stromal cell (MSC) co-infusions. The primary endpoint was to assess the safety of this strategy, as determined by non-relapse mortality. The secondary endpoint was to assess clinical improvement, Col7 (re)expression at the DEJ and quantitative analysis of donor cells dermal chimerism. The trial was approved by the Dutch Central Committee on Research involving Human Subjects (CCMO, the Hague; NL41471.000.13) and was registered in the European Union Clinical Trials Register (EudraCT No: 2012-000605-72). A trial-specific international EB expert advisory panel (listed in acknowledgements) together with the HCT team in UMC Utrecht assessed the eligibility of each patient before inclusion. An independent data safety monitoring board (DSMB) was constituted to periodically review the progress of the trial. The Wilhelmina Children's Hospital of the UMC in Utrecht is a well-established CBT programme in Europe with over 300 CBTs in the last decade.