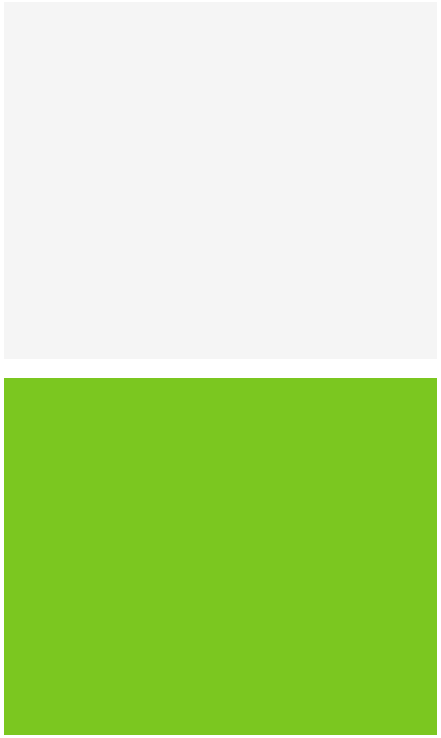
New Therapeutic Solutions for Unmet Clinical Needs



DANIELA COUTO

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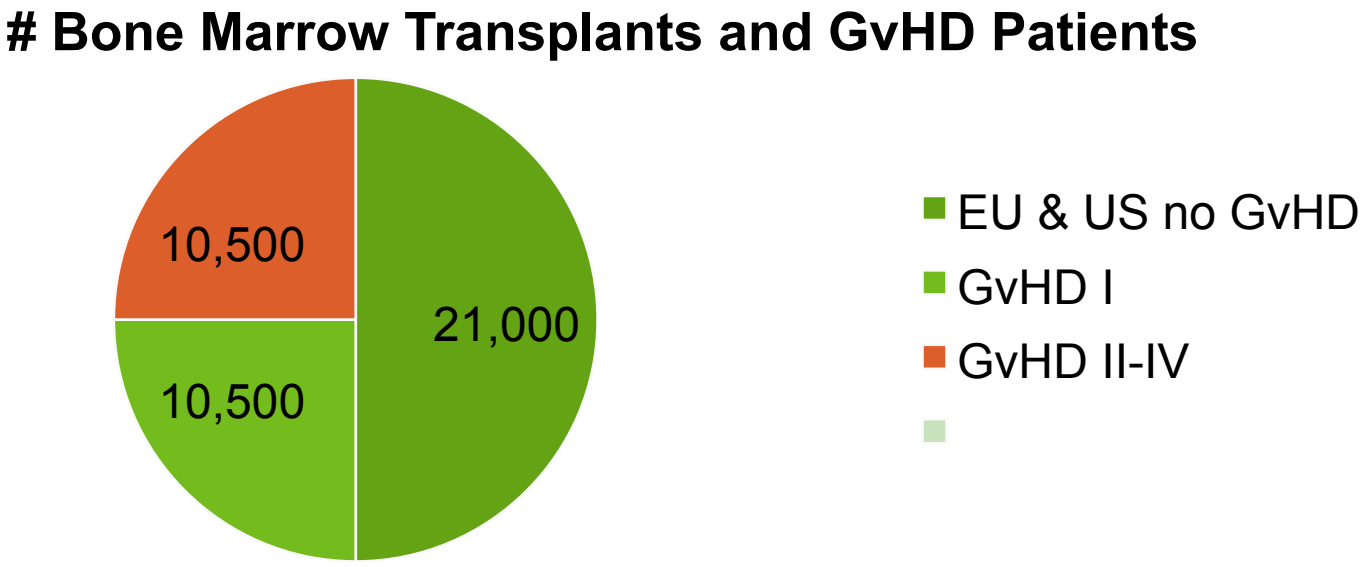
Cell2B Company Fact Sheet

Location:	Cantanhede Portugal
Founding date:	2011
# Employees:	11
Technology:	Animal-origin free matrix for cell isolation and expansion & large-scale bioreactor process (PCT/US2013/030305 & PCT/PT2013/000017) Developed at & licensed from MIT & TÉCNICO LISBOA
Dev. status:	Ready for phase I/II clinical POC for steroid-resistant severe & acute Graft-versus-Host Disease; orphan designation granted

Cell2B: Our Approach to Orphan Designation

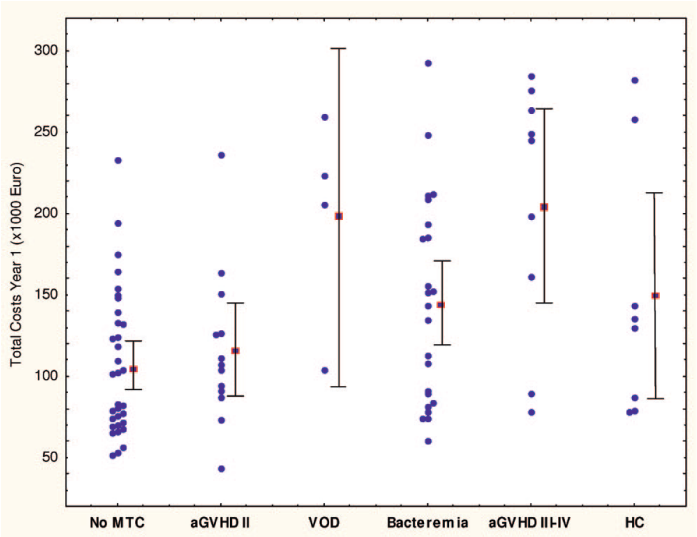
- EMA Granted Orphan Designation for the treatment of GvHD with ImmuneSafe® back in 2013 based on severity of the disease, unmet clinical need, and number of patient with the disease (no more than 0.6 patients in 10,000 persons)
- EMA granted Orphan Designation for the prevention of GvHD in December 2014
- Significant benefit will be re-assessed upon efficacy results
- OUR GOAL: Treat acute cases of GvHD with single treatment <u>and not chronic lifetime treatment</u>

Orphan Designation for GvHD



How much is treatment worth?

Compelling Savings in Treating GvHD



- "Basal" BMT cost

  ~ € 100.000

- aGVHD III/IV cost

  ~ € 210.000

aGvHD III and IV present 70% and 95% mortality rates

Complications Associated with Bone Marrow Transplantation:

- No MTC – no Major Transplant Complications
- aGVHD II – acute GVHG grade II
- VOS – Veno Occlusive Disease of the liver
- Bacteremia
- aGVHD III/IV – acute GVHD III-IV
- HC – hemorrhagic cystitis

How should GvHD treament be reimbursed?

Sustainability in Reimbursing Orphan Diseases

- Orphan diseases typically target small populations
- Restrictions imposed by different reimbursement systems are increasing
- Start reimbursement discussions (HTA bodies) early on for better trial design
  - Should the level of improvement have a direct impact on the reimbursement value?
  - Should cure be reimbursed differently than chronic disease management?

We need better health economic models to reimburse for orphan indications