Improving Stem-Cell Based Cellular Products:

Perspectives from a Patient-Centric Research Organization

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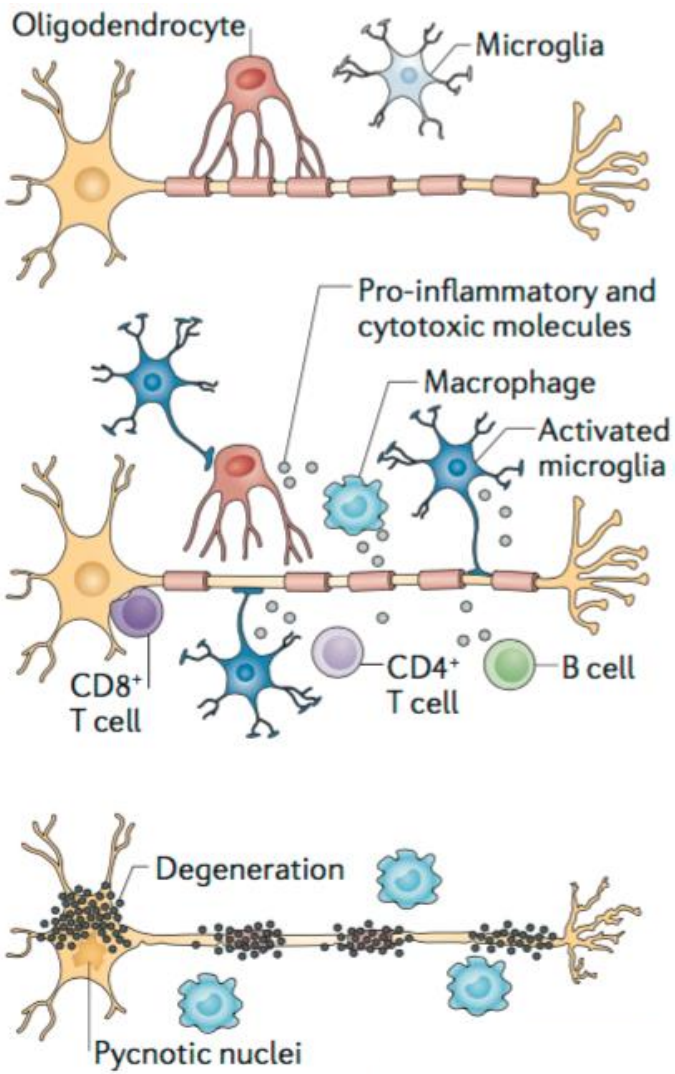
Accelerating Research

to Cure Multiple Sclerosis

What is Multiple Sclerosis?



from Calabrese, *et al.*, 2015

- Autoimmune disease of CNS
- Attacks myelin sheath of nerve axons
- Conduction failure of nerve signals
- Death of nerve cells

- Loss of mobility
- Loss of vision
- Cognitive problems
- Fatigue
- Sleep disorders
- Mental health problems

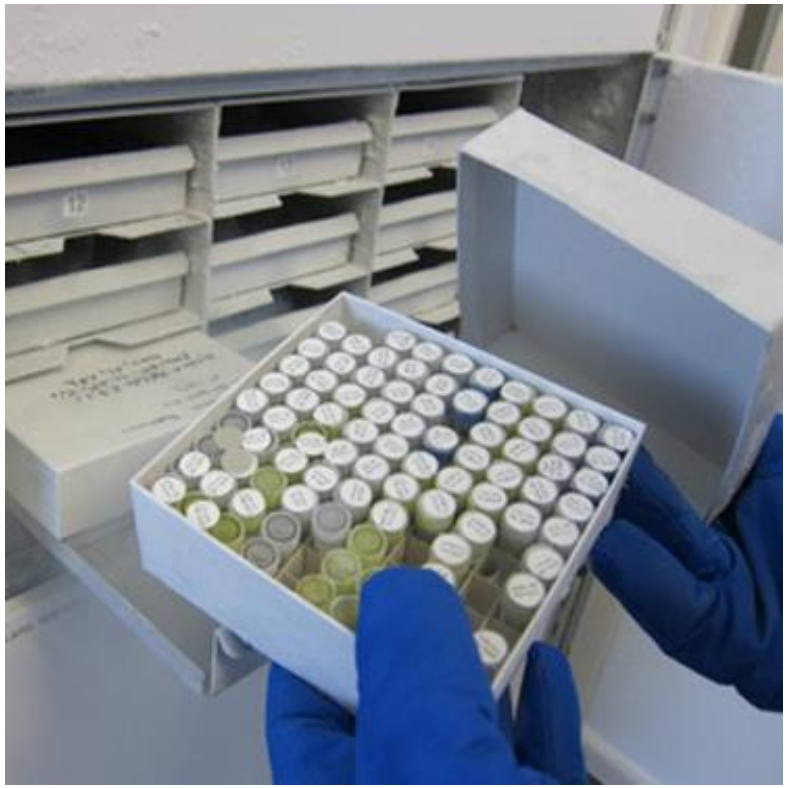
- 14 treatments approved since 1993
- No cure

Background on Accelerated Cure Project

- Founded in 2001
  - MIT-trained engineer diagnosed with MS
  - wanted to accelerated MS research
  - open-science and patient participation principles
- 501(c)3, funded by donors, grants and contracts
- ACP Repository program - since 2005
- iConquerMS™ PPRN - since 2014

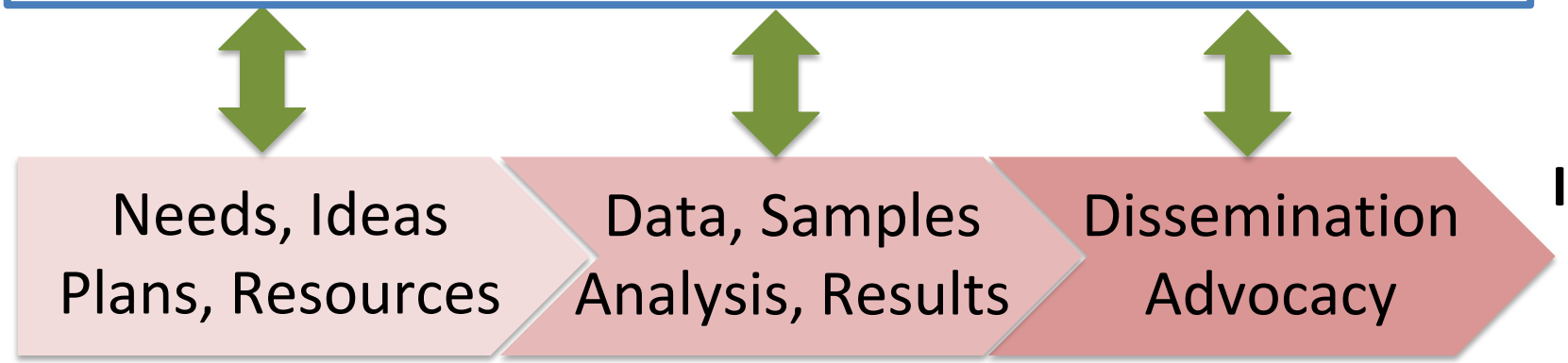
ACP's Programs - Repository

- Biosamples and extensive data from 3,200+ consented individuals at 10 MS Clinics (funded by ACP)
- Commercial biorepository partner for processing/storage
- Available to researchers worldwide (100+ studies, so far)
- Mandated data return creates virtual collaborations
- ACP manages samples/data distributions & returned data



ACP's Programs - iConquerMS™

iConquerMS™: People-Powered Research Network bridged to Researchers and Other Stakeholders



- ~4,000 participants*, growing daily
- Funded by PCORI as part of PCORnet
- Governed by majority of PwMS - the experts
- Research portfolio developing rapidly

OPEN SCIENCE driven by People with MS

*registered & consented + 1,200 e-mails (1,300 FB; 2,100 twitter)

Regenerative Medicine Opportunities in MS

- "Confused" Immune System
  - Reboot with stem cells (hemopoietic, mesenchymal)
- Damaged Myelin Sheath
  - Regenerate oligodendrocytes (mesenchymal SCs, iPSCs)
- Lost Nerve Cells
  - Regenerate nerve cells and make right connections (mesenchymal SCs, iPSCs)

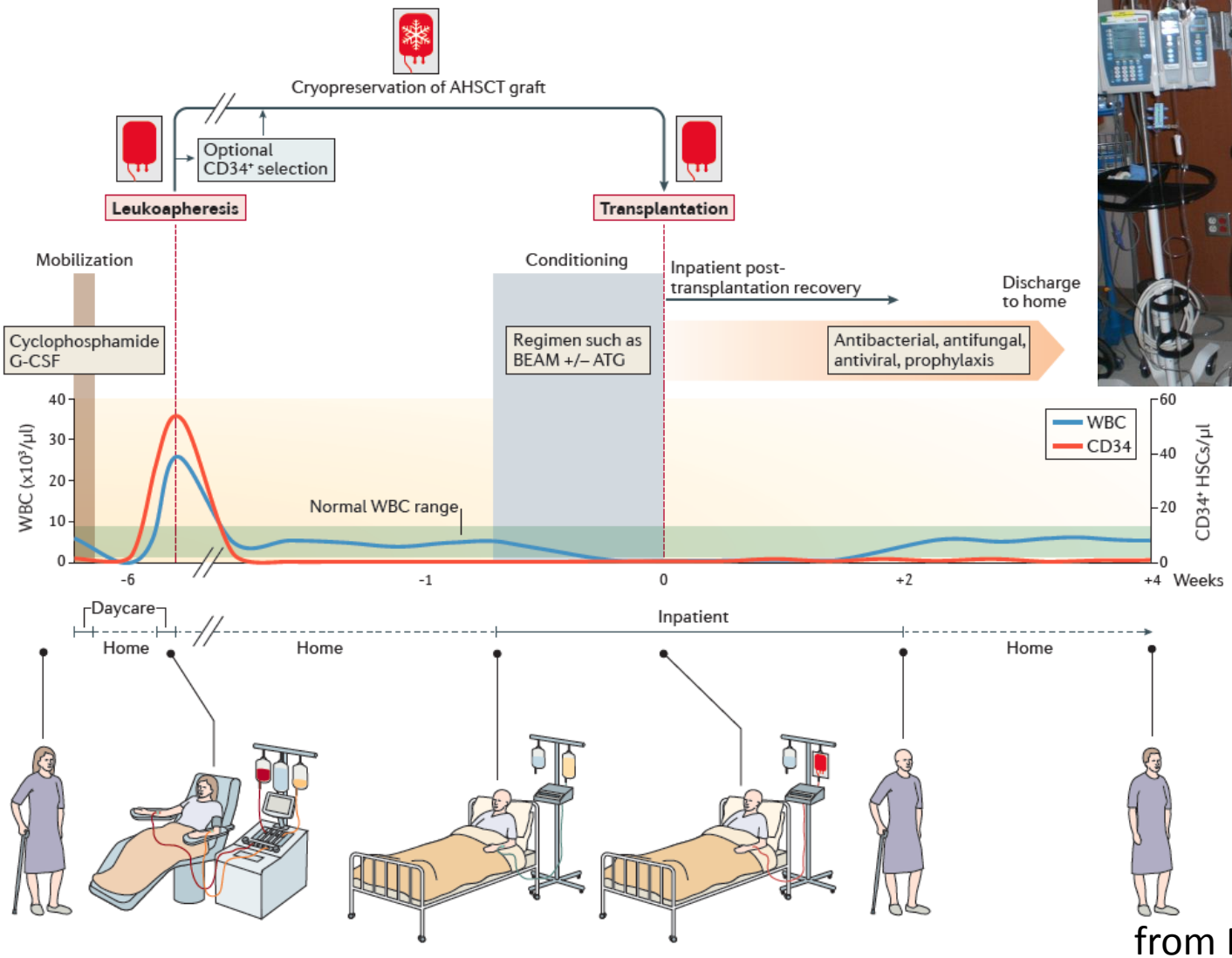
50+ clinical studies registered on clinicaltrials.gov

16 US studies

12 US academic studies completed or no longer recruiting

4 US companies with AMSC studies "recruiting"

Autologous Hemopoietic SCs for MS

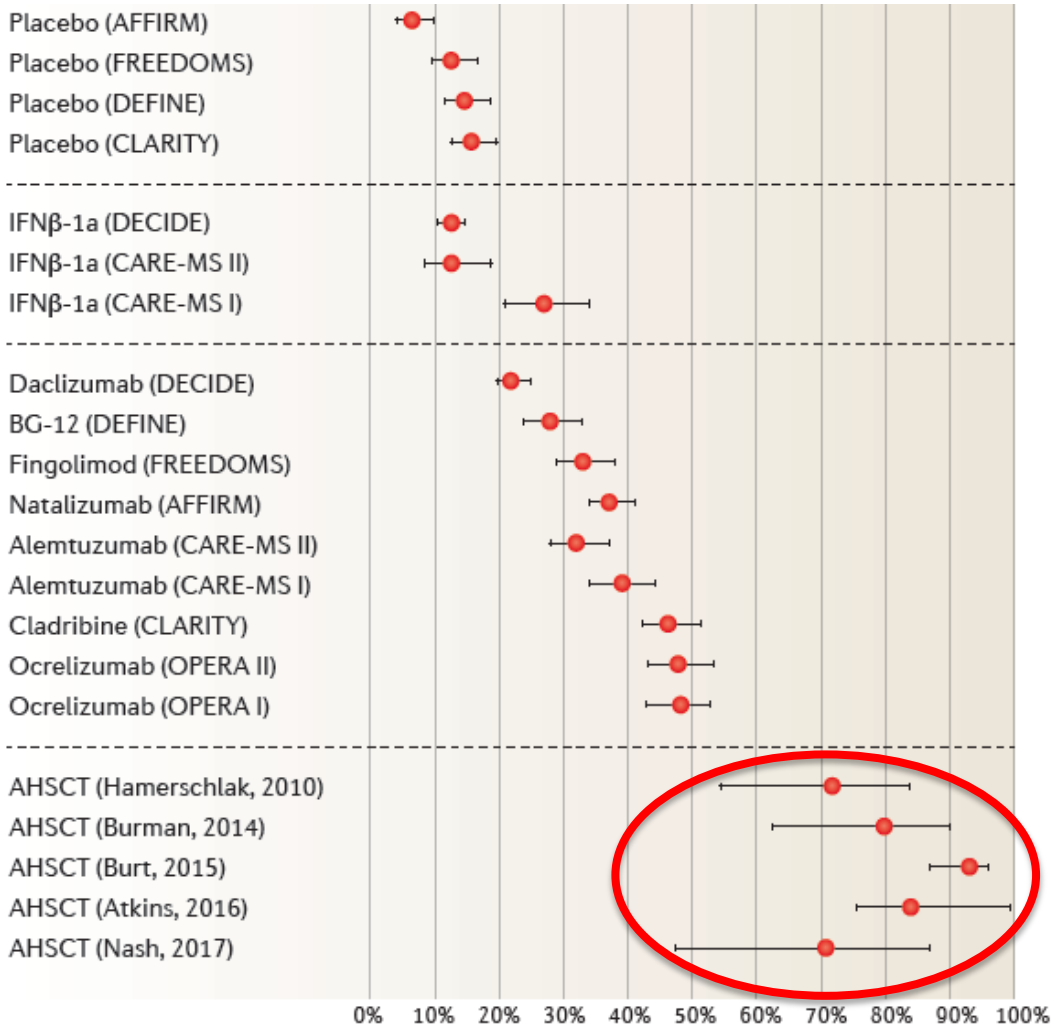


Dave Bexfield
ActiveMSers &
iConquerMS™

from Muraro, *et al.*, 2017

Autologous Hematopoietic SCs for MS

% of Patients with No Evidence of Disease Activity at 2 years



Positive trials have led to plans for BEAT-MS clinical study

from Muraro, *et al.*, 2017

Key to Trials and Routine Treatment

Material with Right Properties

In the Right Place

At the Right Time

At the Right Concentration

Improving SC-Based Products

Role of Patients and Advocacy Organizations

- Patients are the source of treatment material
- Educate patient and caregiver communities through networks and advocacy organizations
- Emphasize the need for high-quality clinical trials
- Bridge the expectation-reality gap driven by hope
- Include people affected by the disease in the entire continuum from study concept to impact on patients
- Protocol development, study participation, results dissemination, approvals, reimbursement

Improving SC-Based Products

Role of Biorepositories

- Research on the properties of cells at various stages is critically important to developing/improving products
- Harvesting, destroying, stimulating, post-treatment
- Need centralized biorepository for processing, storage and distribution of materials/data for research
- Need neutral management and oversight of:
  - activities of biorepository;
  - study approval and material/data distribution process; and,
  - returned data from research studies


Accelerated
Cure
project
for multiple sclerosis


iConquerMS
TM

Thank You