



Clinical Trial Data Sharing

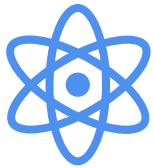
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Fuku Biotech



Why Share Data?

Reasons for Data Sharing



Promote scientific
discoveries



Informed
decision-making



Enhance Clinical
Trial Transparency



Promote a sense
of collaboration

Challenges



Legal Concerns



Ethical Concerns



Industry Reluctance



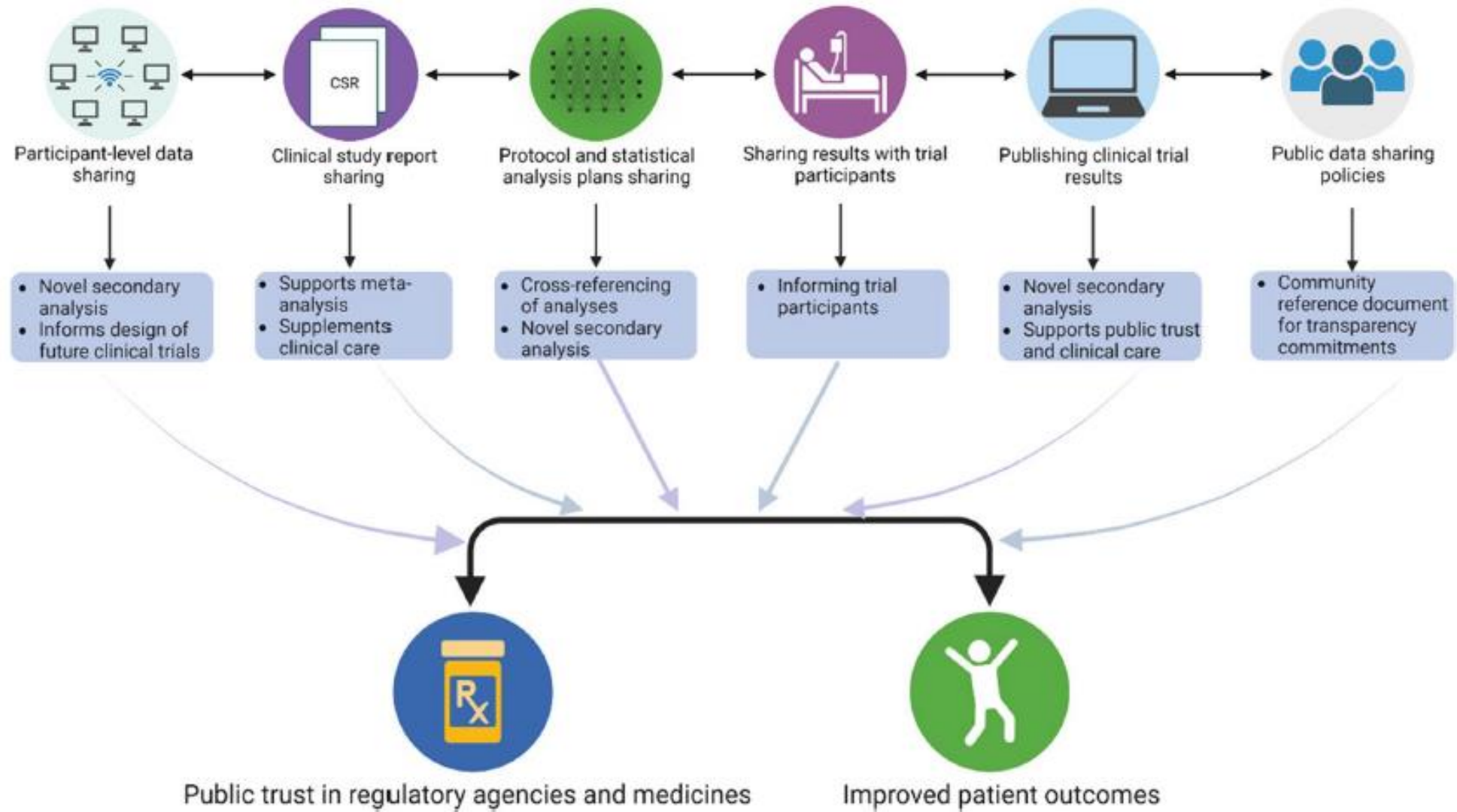
Intellectual Property Issues



Considerations for Sharing Data

- Balance Control with Transparency
 - Stakeholders?
 - Academics
 - Industry
 - Patients-Families-Foundations
 - Need for Prospective Governance
 - Public Registries
 - Data Standardization
 - Open Access Models-Data Security
- 





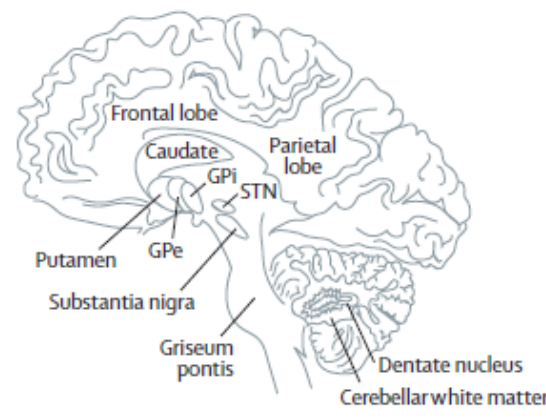


Case Study

Davunetide:
Progressive Supranuclear Palsy

Progressive Supranuclear Palsy (PSP)

- > Rare, neurodegenerative disease (5 to 7 cases per 100,000)
- > Typically manifests in individuals over 60 years old
- > A progressive, degenerative disease involving the brain stem, basal ganglia, cerebellum
- > Clinical symptoms (movement problems, cognitive impairment) apparent result of the underlying tau pathology in the brain region controlling those functions



Steele JC, Richardson JC, Olszewski J. 1964 Arch Neurol;10: 333–59.

Williams and Lees; *Lancet Neurol* 2009; 8: 270–79

Study AL-108-231 (NCT 01110720)

Phase II/III study in PSP

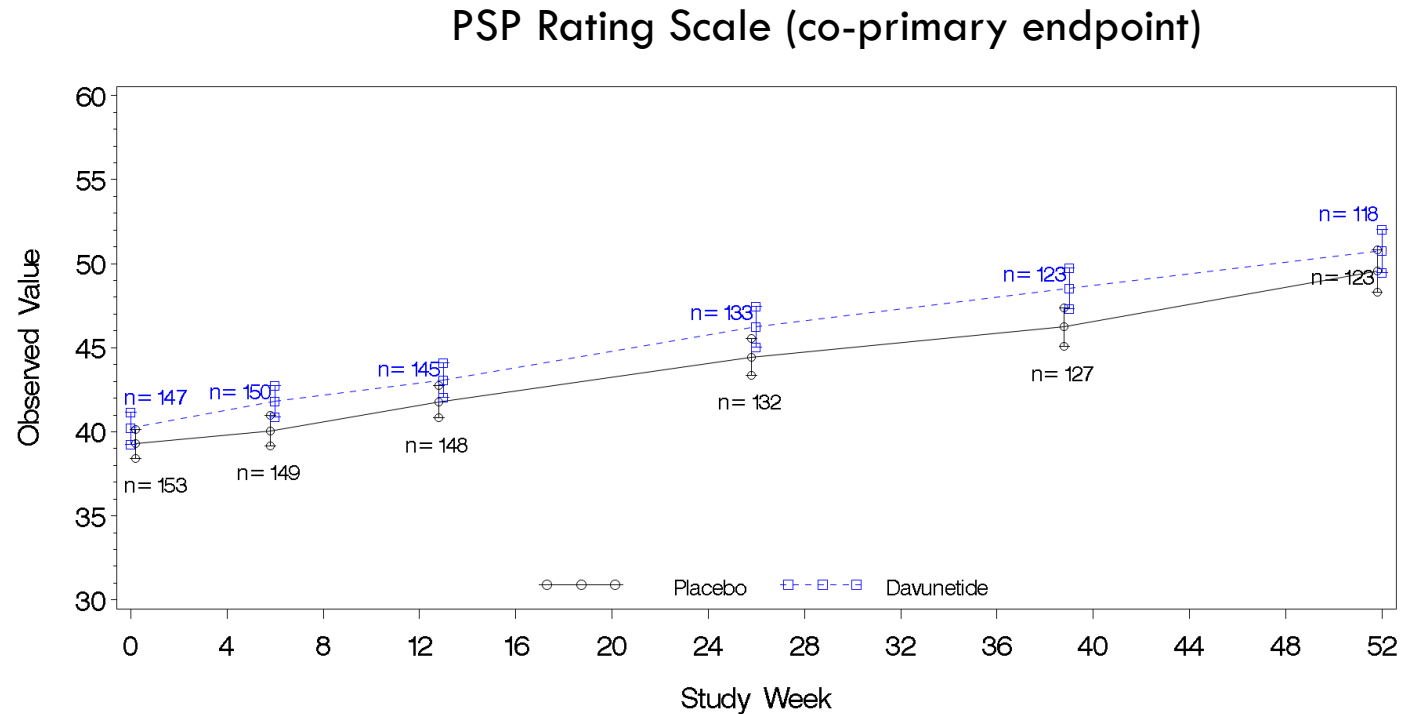
- Recruited 313 patients
- 1:1 active-to-placebo
- Treatment for 1 year
- 47 clinical sites in US, Canada, Australia, Germany, UK and France

Clinical Endpoints

- Safety (adverse events, con meds)
- Efficacy (disease severity, daily living, cognitive, mood)
- Volumetric MRI
- CSF biomarkers
- DNA (tau genotype)

AL-108-231 Results

Active, no different from placebo on primary or secondary endpoints



Valid study: PSP disease progression over 12 months as expected

What happened next?

- Steering Committee Charter executed when the study started
 - Responsible for primary publication
 - Review post-study requests for data and/or samples (company retained right to review any publication or presentation to preserve IP)
- Published results
- De-identify samples (blood, plasma, DNA, CSF) and clinical data
- Data and samples transferred to UCSF

Primary publication: Lancet Neurology 13(7), 676-685 (2014)

Separate coordinating Investigator agreement covered roles & responsibilities

Need to ensure Informed Consent allowed for clinical data and sample use (!)

Impact

Every subsequent PSP study accessed and used the davunetide AL-108-231 data: power calculations, outcome measure performance

1. Mov Disord 31(10), 1574 (2016). Minimal clinically important worsening on the PSP-RS
2. Parkinsonism Relat Disord 28, 41 (2016). Predicting disease progression in PSP in multicenter clinical trials
3. Mov Disord 31(5), 742 (2016). Power calculations and placebo effect for future clinical trials in PSP
4. Parkinsonism Relat Disord 28, 29 (2016). Clinical correlates of longitudinal brain atrophy in PSP
5. Neurology 87(19), 2016 (2016). Progression of brain atrophy in PSP and CBS over 6 months and 1 year
6. Mov Disord 32(6), 842 (2017). Longitudinal magnetic resonance imaging in PSP: a new combined score for clinical trials
7. Neurology 90(4), e273 (2018). CSF neurofilament light chain and phosphorylated tau-181 predict disease progression in PSP
8. Parkinsonism Relat Disord 60, 138 (2019). Severity dependent distribution of impairments in PSP and CSB: interactive visualizations
9. Mov Disord 37(6) 1265 (2022). Modified PSP-RS for virtual assessments
10. JAMA Neurol 81(3), 295 (2024). Concomitant medications for PSP: a secondary analysis of a randomized clinical trial
11. Mov Disord 39(8), 1329 (2024). Magnetic resonance imaging measures to track atrophy progression in PSP in clinical trials





Lessons Learned

- Plan Prospectively
- Integrated part of study and program
- Allocate resources

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Associations-Foundations

- CurePSP
- AFTD



Thank you

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