



From Outsourced to Open Source: Denali's R-
Driven Transformation in Biometrics

PHUSE SDE: July 23

- Nicole Jones, MPH
 - Principal Data Scientist
 - Study Lead
 - R Package Validation Lead
 - Shiny Developer
 - Posit Admin



All the views shared in this presentation are my own and do not reflect the opinions of my company

OVERVIEW

- What We Did
- How We Did It
- Where We're Headed

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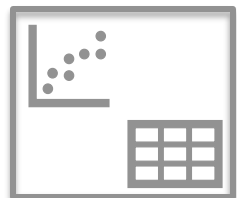
What Did We Do?



Our First Successful BLA Filing in May 2025
via AA Pathway



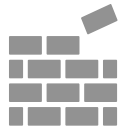
2 Clinical Study Reports (Natural History + Phase I/IIB)
1 Integrated Summary of Efficacy
1 Biomarker Reference Range Study



~1000 In-House TLFs + Datasets (R)
~640 Vendor Produced TLFs + Datasets (SAS)

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Our Foundation



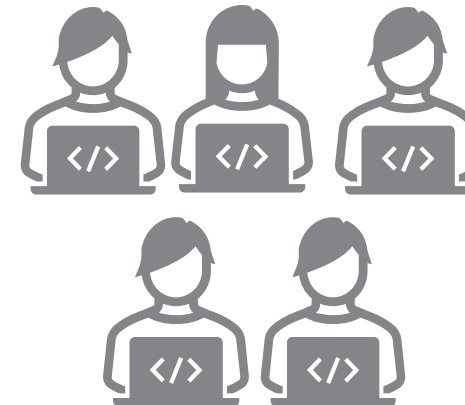
Internal Exploratory Analyses
In-House (R)



Regulatory Work/Reporting Events
Outsourced (SAS)



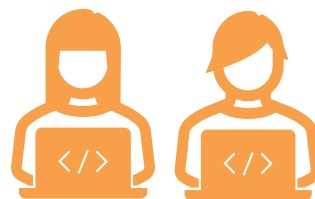
Our Goal



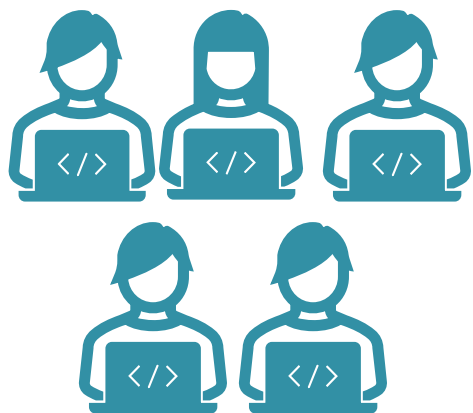
Build an In-House Team
capable of supporting our
regulatory needs



The Road to Achieving Our Goal



Internal R Team



Contractors (SAS)



CROs



The Road to Achieving Our Goal





The Road to Achieving Our Goal





The Road to Achieving Our Goal



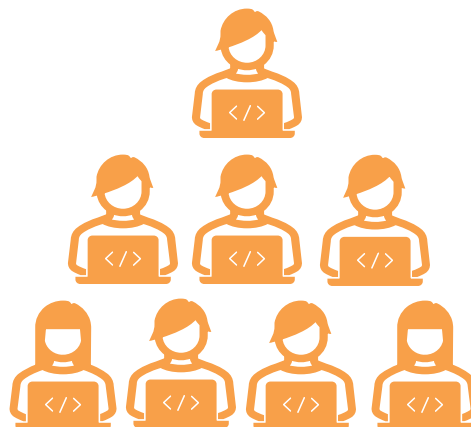
**An extensive library of
open-source packages**



The Road to Achieving Our Goal



SAS
Contractors



Internal R Team



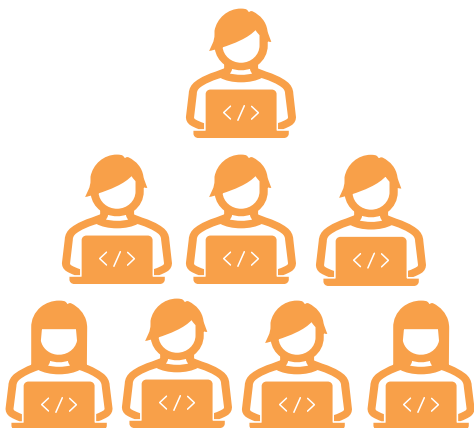
CROs



R Contractors



Key Drivers of Success – Our Team



Our Internal R Team

Experienced R Programmers

- 3 pharmaverse contributors
- 3 R Pilot submission contributors
- Programmers with extensive experience using R to support clinical trials



Key Drivers of Success – Systems Investments



Statistical Computing Environment

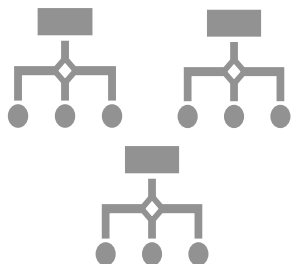
- Cloud based
- Customized GxP R environment



Key Drivers of Success – Process Investments

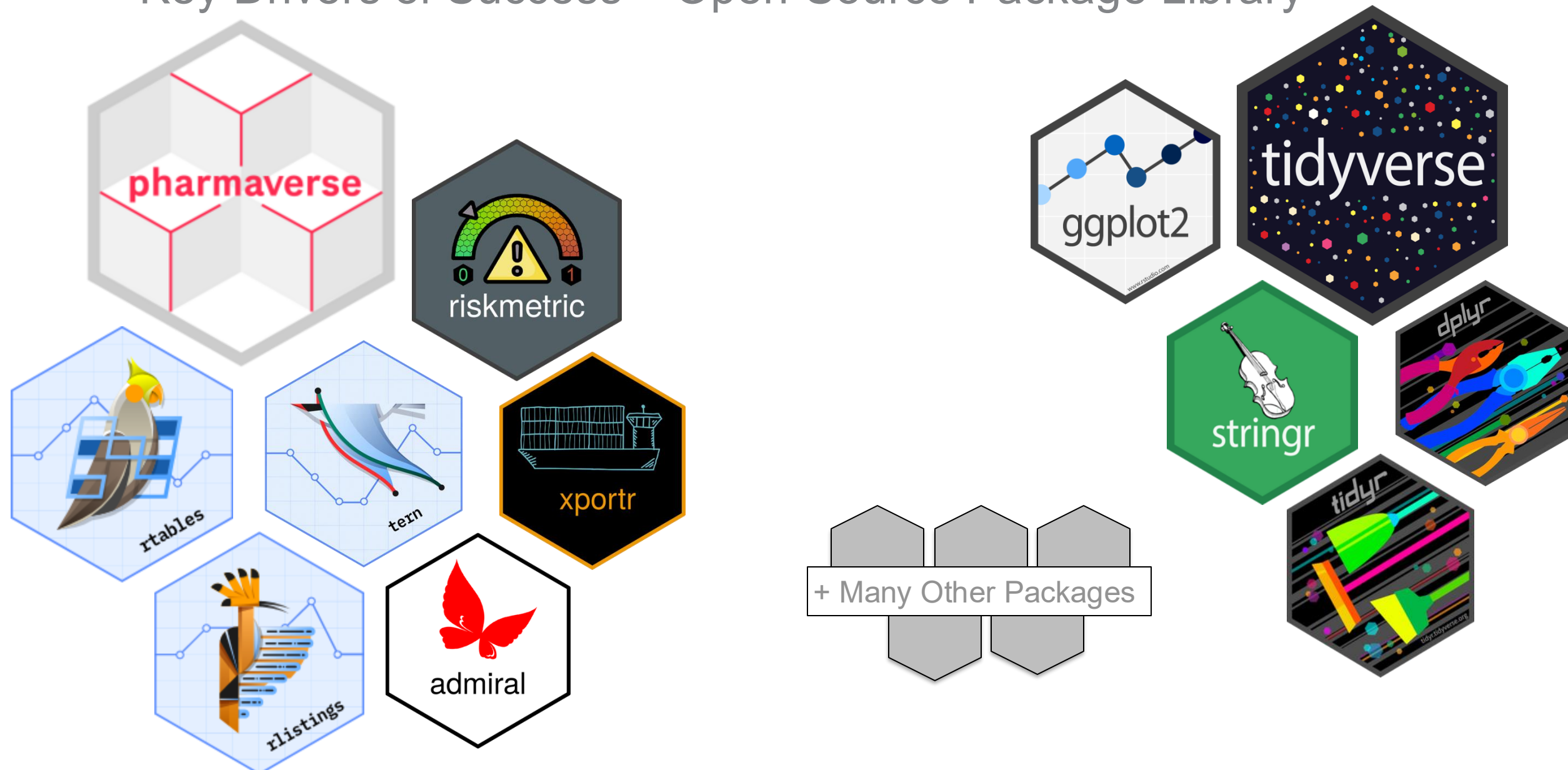


R Package Validation Process



Parameterization of Programs

Key Drivers of Success – Open-Source Package Library





Lessons Learned



Start your define package early

CRO generated package with
significant oversight, management
and editing from our team



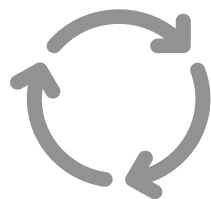
Lessons Learned



Vendor oversight was a
time consuming task



Lessons Learned



Agility of our In-House Team



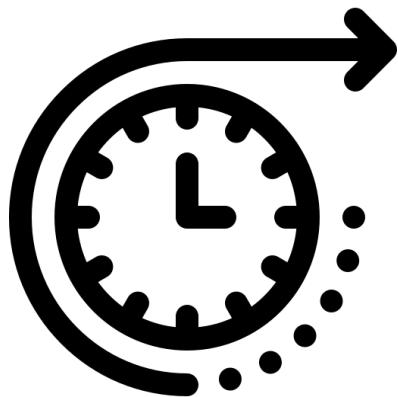
Lessons Learned



SMEs appreciated the flexibility
of generating graphics in R



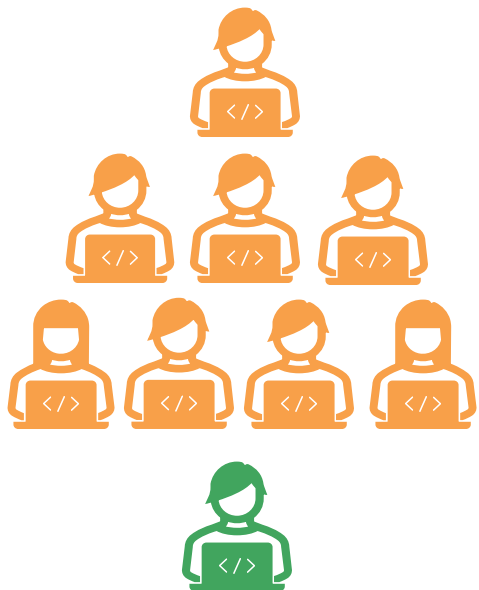
Lessons Learned



The future is R



Lessons Learned



Our Team can do this!

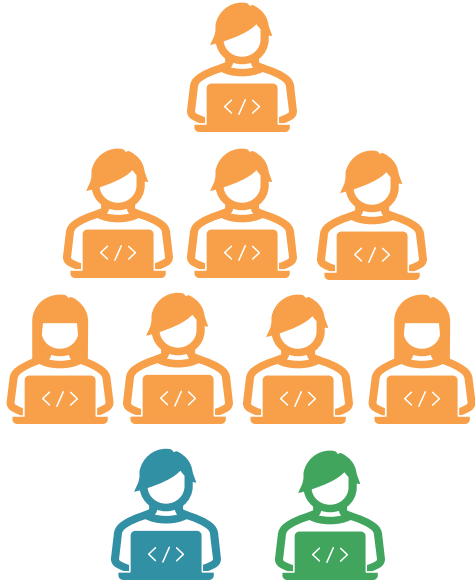
~ 60% of Outputs
Generated In-House

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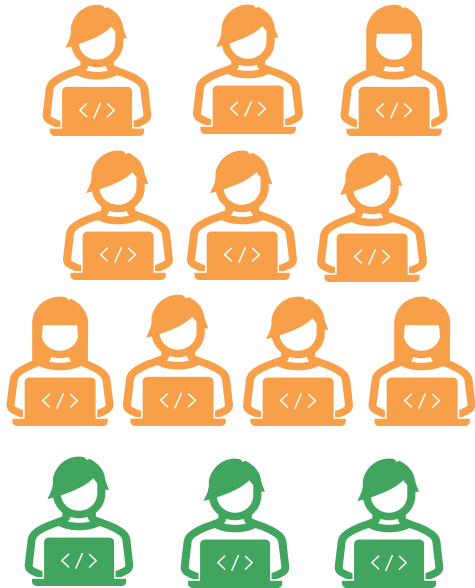
Where are We Headed



Continue to Invest in
our Internal R Team



Where are We Headed



Continue to Invest in
our Internal R Team



Where are We Headed

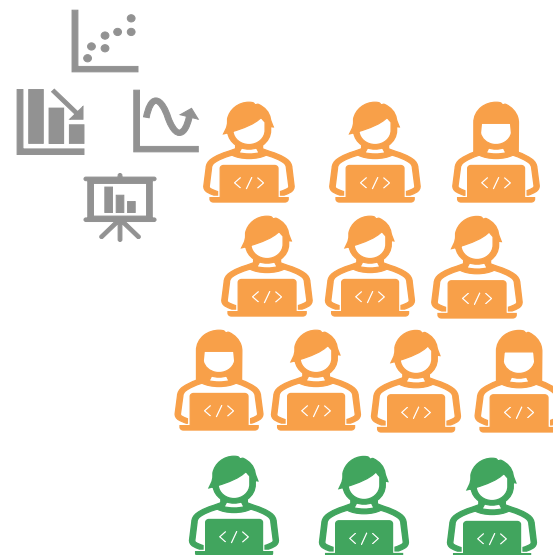




Where are We Headed



Move all regulatory
work in-house



ACKNOWLEDGEMENTS + CONTACT

Thank you to my internal team that made this possible:

AJ Karunakara

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Michael Thorpe

Nolan Steed

Kyle Ireton

Kenneth Chin

If you have questions or would like to talk more feel free to reach out:

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BACKUPSLIDE

A	B	C		D	E	F		G	H	I	J	K	L	M
output_number	type	Title	footnote	Population	output_name	program	format	ant_filter	ant_var	by_var	extra_args	pop_filter		
1	Figure	Spaghetti Plot of Biomarker 1	Sample Footnote 1	Full Analysis Population	f_bm_BIOM1_AVAL_TRT01A	f_bm	pdf	BIOM1	AVAL	TRT01A	x_var=ADY	FAS		
2	Figure	Geometric Mean by Visit Biomarker 1	Sample Footnote 2	Full Analysis Population	f_bm_gm_BIOM1_FCHG_TRT01A	f_bm_gm	pdf,png	BIOM1	FCHG	TRT01A	x_var=ADY	FAS		
3	Figure	Spaghetti Plot of Biomarker 2	Sample Footnote 3	Full Analysis Population	f_bm_BIOM2_AVAL_TRT01A	f_bm	png	BIOM2	AVAL	TRT01A	x_var=ADY	FAS		
4	Table	Geometric Mean by Visit Biomarker 2	Sample Footnote 4	Full Analysis Population	f_bm_gm_BIOM2_FCHG_TRT01A	f_bm_gm	pdf,png	BIOM2	FCHG	TRT01A	x_var=ADY	FAS		
5	Table	Descriptive Statistics of Biomarker 1	Sample Footnote 4	Full Analysis Population	t_bm_summary_BIOM1_TRT01A	t_bm_summary	pdf,docx	BIOM1		TRT01A		FAS		
6	Table	Descriptive Statistics of Biomarker 2	Sample Footnote 6	Full Analysis Population	t_bm_summary_BIOM1_TRT01A	t_bm_summary	pdf,docx	BIOM1		TRT01A		FAS		

Questions?



THANK YOU