CDISC Italian User Network 2020

Milan, Italy | 7 October 2020

Efficiently Leveraging Your RWD Investment Across the Product Life Cycle

Stijn Rogiers, Pr. Industry Consultant, SAS Global Health and Life Sciences Practice

Real World Evidence

A Hot topic today…

Maximize product value beyond efficacy and safety

Society

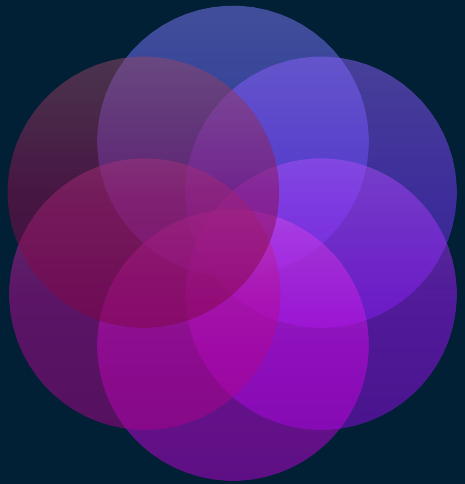
Equal access to healthcare
Return to work
Caregiver burden
Productivity

Payer

Comparative effectiveness
Cost-utility
Budget Impact
Long-term Outcomes

Patient

Morbidity, Mortality
Symptoms, Functionality
Preferences, Quality of Life



Economic

Direct/Indirect Cost
Cost:Outcomes
Cost Consequences
Resource Utilization
QALY

Provider

Effectiveness
Tolerability
Safety
Compliance
Adherence
Convenience

Clinical

Efficacy
Safety
Morbidity
Mortality

Real-world data (RWD) and real-world evidence (RWE) are playing an increasing role in health care decisions

US FDA

1. Publication and Guidance documents incl. submission
2. Framework for FDA's RWE Program (Dec 2018)

HMA-EMA Joint Big Data Task Force

1. Publications incl. Big Data Steering Group workplan for 2020-21
2. Summary Report (Feb 2019)

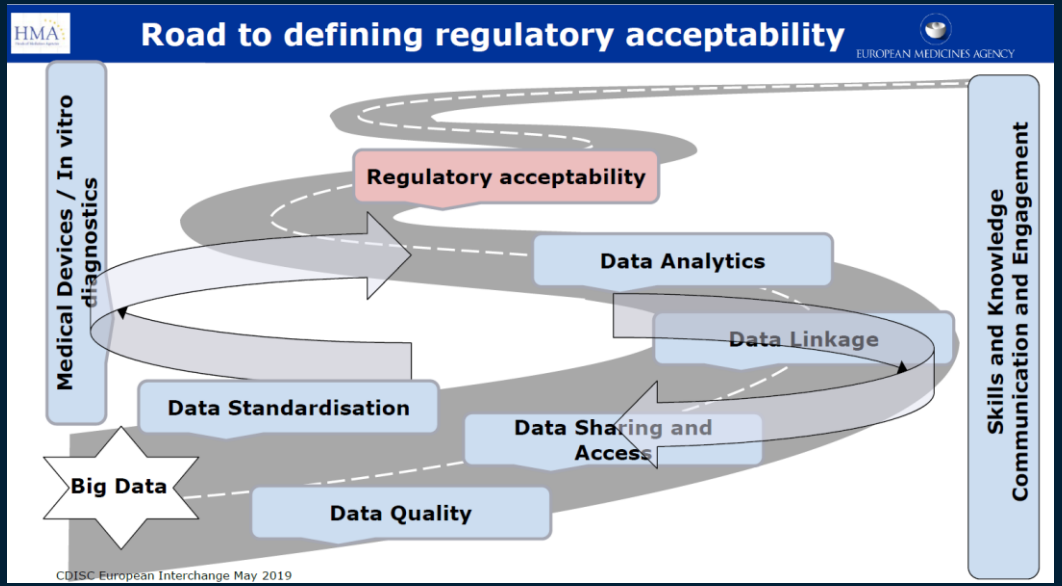
National Academies of Science (US)

1. Real-World Evidence Generation and Evaluation of Therapeutics (Workshop 2017)
2. Examining the Impact of Real-World Evidence on Medical Product Development I. Incentives (Feb 2018)
3. Examining the Impact of Real-World Evidence on Medical Product Development II. Practical Approaches (Jul 2018)

The Academy of Medical Sciences (UK)

1. Real-World Evidence Generation Scoping Roundtable (Jan 2018)
2. Next steps for using real world evidence (May 2018)

RWE stream @ CDISC EU Interchanges 2019 & 2020



Expanding the Use of Real-World Evidence in Regulatory and Value-Based Payment Decision-Making for Drugs and Biologics (Aug 2019)

Conclusion

Real-world evidence generated from the application of appropriate research methods and analysis of data derived from clinical, claims, and patient-facing software and systems can play a significant role in improving and modernizing both the drug evaluation and approval process and new value-based payment arrangements in the United States. Progress has already been made by the FDA in establishing a framework for the use of real-world evidence for regulatory decision-making. Significant progress has also been made by ONC and CMS in advancing interoperability and use of common standards in systems, particularly through the development of recently proposed rules. Recent efforts by HHS to promote regulatory reforms can also play a key role in modernizing evidence development.

Regulatory and payment decision-making will be informed and improved by improving regulatory clarity; increasing support for real-world evidence activities; enabling access to and analysis of data; improving its reliability and relevance; expanding pilot and demonstration projects; clearing regulatory barriers; building capacity within federal agencies; and promoting collaboration among payers, regulators, and other key stakeholders around the generation and use of real-world evidence. These steps will ultimately help to accelerate the availability of safe, effective, and affordable medicines for patients in need.

Reference: https://bipartisanpolicy.org/report/expanding-the-use-of-realworld-evidence-in-regulatory-and-value-based-payment-decision-making-for-drugs-and-biologics/

How to ensure that novel analytic methods are fit for decision-making (Oct 2019)

News 02/10/2019

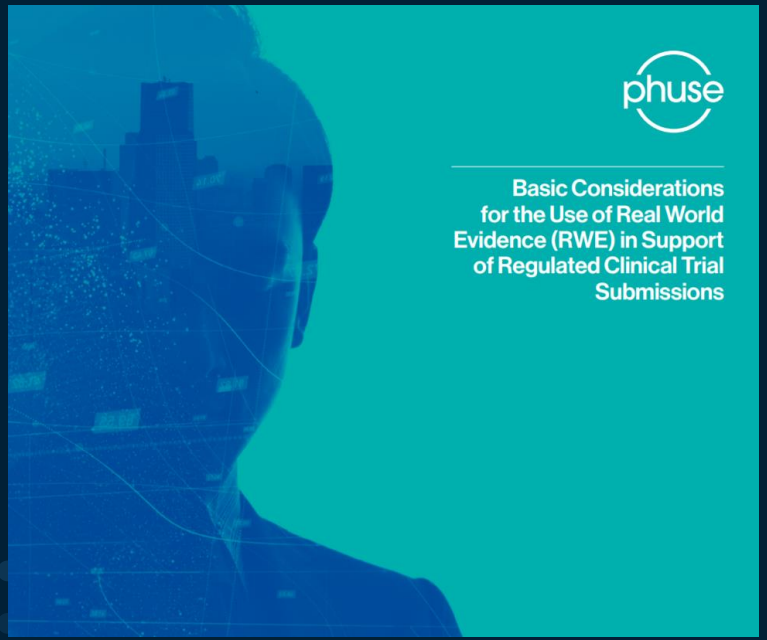
The past decade has seen the increased generation and availability of new data sources such as real-world evidence, as well as patient-level data from completed randomised clinical trials. While these data provide an opportunity to learn more about a medicine's benefits and risks, and can complement the main body of evidence coming from randomised clinical trials, they will not necessarily translate into credible evidence for regulators and other decision-makers in the absence of adequate statistical methods to extract, analyse, and interpret them.

In an article published in Clinical Pharmacology & Therapeutics, regulators and academics explain how proper methodological validation can ensure the credibility of these data sources and allow authorities to rely on them to draw reliable scientific conclusions. The article is co-signed by a number of EMA staff members, academics

https://www.ema.europa.eu/en/news/how-ensure-novel-analytic-methods-are-fit-decision-making

Real World Evidence

PHUSE Paper (Jul 2020)



Using RWE may provide advantages in cost, cohort sizes and ethical considerations.

This PHUSE paper attempts to collate all related information such as sources of RWD (Real-World Data), privacy laws and use cases in one document which can act as a reference point for individuals or companies who wish to design, conduct, and submit studies using RWE (Real-World Evidence).

It includes FDA's current direction and guidance as of the date of this publication.

PHUSE RWE Paper

Real World Evidence

Examples

Example (Jan,2018)

J&J performed a **pragmatic** real-world trial, a randomized trial that used measures that are collected in clinical practice

*"…INVEGA SUSTENNA® (paliperidone palmitate), a once-monthly schizophrenia treatment, is the first and only antipsychotic to have the U.S. Food and Drug Administration (FDA) **approve the inclusion of real-world data in its product labeling**"*

https://www.jnj.com/media-center/press-releases/landmark-schizophrenia-data-that-brings-hope-in-breaking-the-cycle-of-hospitalization-and-incarceration-receives-fda-approval-for-inclusion-in-invega-sustenna-paliperidone-palmitate-label

What's scary, and appealing, about real-world evidence (StatNews 2019)

Reference: "*Real World Evidence in Clinical development, life cycle management and/or repurposing*"
(Julijana Dukanovic, Andrew Leary – Dr. Regenold GmbH, SAS HLS Exec Forum Basel, 25 June 2019)

Example (Apr,2019)

*"…**The approval is based on RWD** from electronic health records and post-marketing reports of Ibrance™ in male patients sourced from three databases: IQVIA Insurance database, Flatiron Health Breast Cancer database and the Pfizer global safety database"*

The new label reads: "Based on limited data from postmarketing reports and electronic health records, the safety profile for men treated with IBRANCE is consistent with the safety profile in women treated with IBRANCE."

Pfizer Uses EHR Data to Support Expanded Indication for Breast Cancer Drug

https://www.raps.org/news-and-articles/news-articles/2019/4/pfizer-uses-ehr-data-to-support-expanded-indicatio

Reference: "*Real World Evidence in Clinical development, life cycle management and/or repurposing*" (Julijana Dukanovic, Andrew Leary – Dr. Regenold GmbH, SAS HLS Exec Forum Basel, 25 June 2019)

Stakeholders and Use Cases

Epidemiology

Medical Affairs

Pharmacovigilance

HEOR

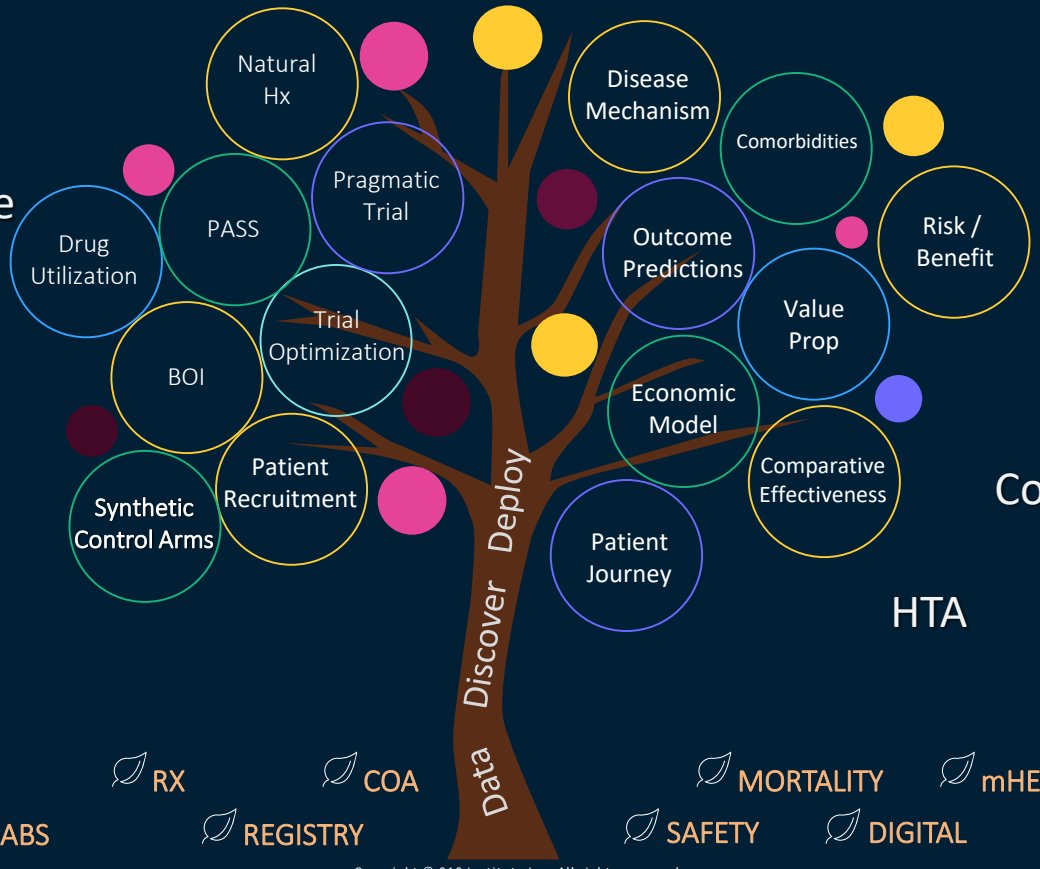
Regulatory Affairs

Market Access

Commercial

R&D

HTA



PRO CLAIMS RX COA MORTALITY mHEALTH CONSUMER

EMR LABS REGISTRY SAFETY DIGITAL SOCIAL MEDIA

Synthetic Control Arms

Link In Post - PHUSE EU Connect 2020

Real World Evidence

CDISC

CDISC RWD Connect

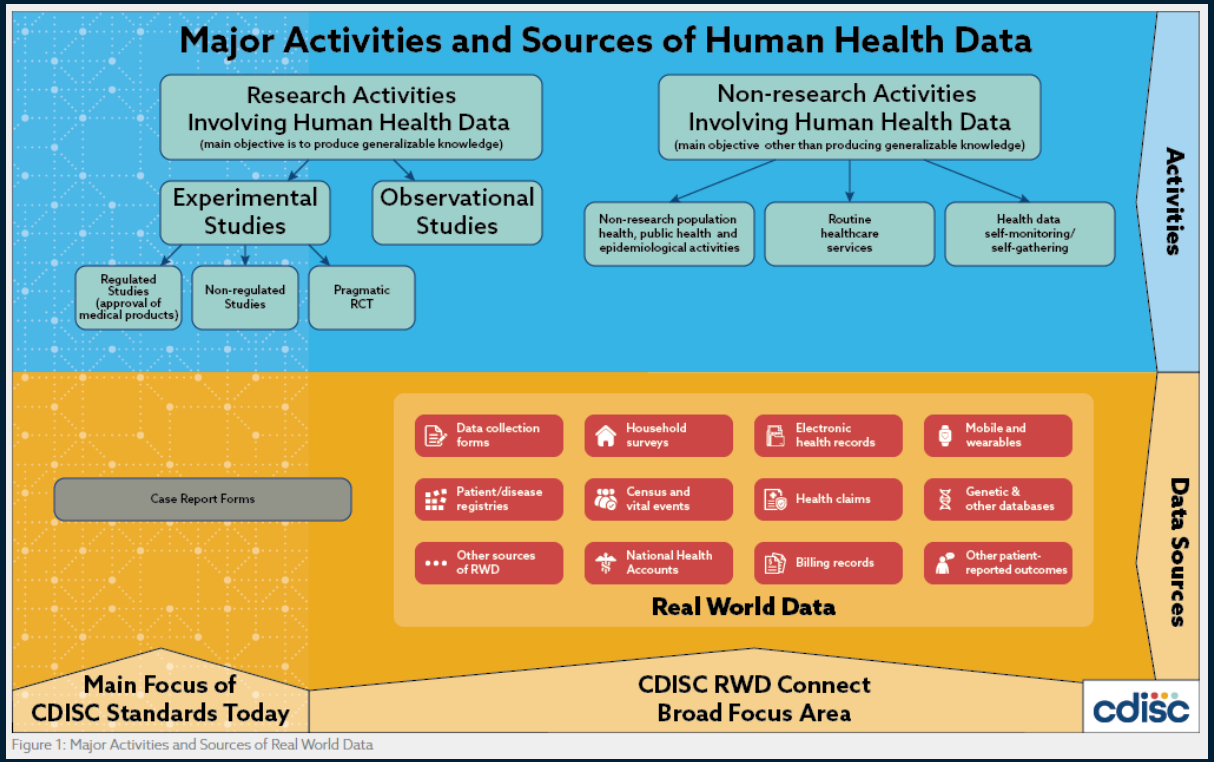


Figure 1: Major Activities and Sources of Real World Data

https://www.cdisc.org/standards/real-world-data

Call for Abstracts (incl. RWE stream)

CDISC EU Interchange 2021

Stijn Rogiers
E3C Member and Principal Industry Consultant, Global Health and Life Sciences Practice, SAS

Earlier this year we made a drastic decision and changed the EU CDISC Interchange 2020 from an in-person (Berlin) conference to a virtual event in a matter of weeks/days.

It was a real challenge, but we did it! What a great CDISC E3C/ CDISC team effort, considering the short deadline we faced. **You can only do this when you are well-prepared and act as ONE team.**

Approximately six months later, the E3C team met virtually for two half days (10-11 Sep) to start planning the **EU interchange 2021** .

As COVID-19 will continue to impact our lives for a while, the logical decision was made by CDISC and the CDISC E3C team to start preparing for another **virtual event** (*most probably last week of April 2021*).

CDISC E3C **"Godfather" Joerg Dillert** kept an eye on the Virtual E3C meeting and noticed that the preparation with new lead **Nick De Donder** and co-lead **Sujit Khune** ran very smoothly.

Meeting topics included feedback from this year CDISC 2020 Europe Virtual Interchange, new ideas and trends for the upcoming event, the new virtual platform and its capabilities, **COVID-19**, **CDISC-360**, keynote presentations), CDISC (virtual) trainings, the collaboration with CDISC (country) teams and many more.

- The **call for abstracts will open 9 Oct 2020**. Check out the CDISC website and follow **CDISC** and E3C team members on social media to get all latest updates on this upcoming event.
- Final **submission deadline is set for 8 Jan 2021.** We encourage you to share your expertise by submitting an abstract. Don't forget... **Sharing Is Caring!**

Are you interested in joining the CDISC E3C team ?

We are always looking for enthusiastic industry talents from Pharma, CRO, Academia,

Technology, or Regulatory to strengthen our diverse team.

Stay tuned for an official CDISC announcement soon.

Don't hesitate to **contact us** in the meantime.

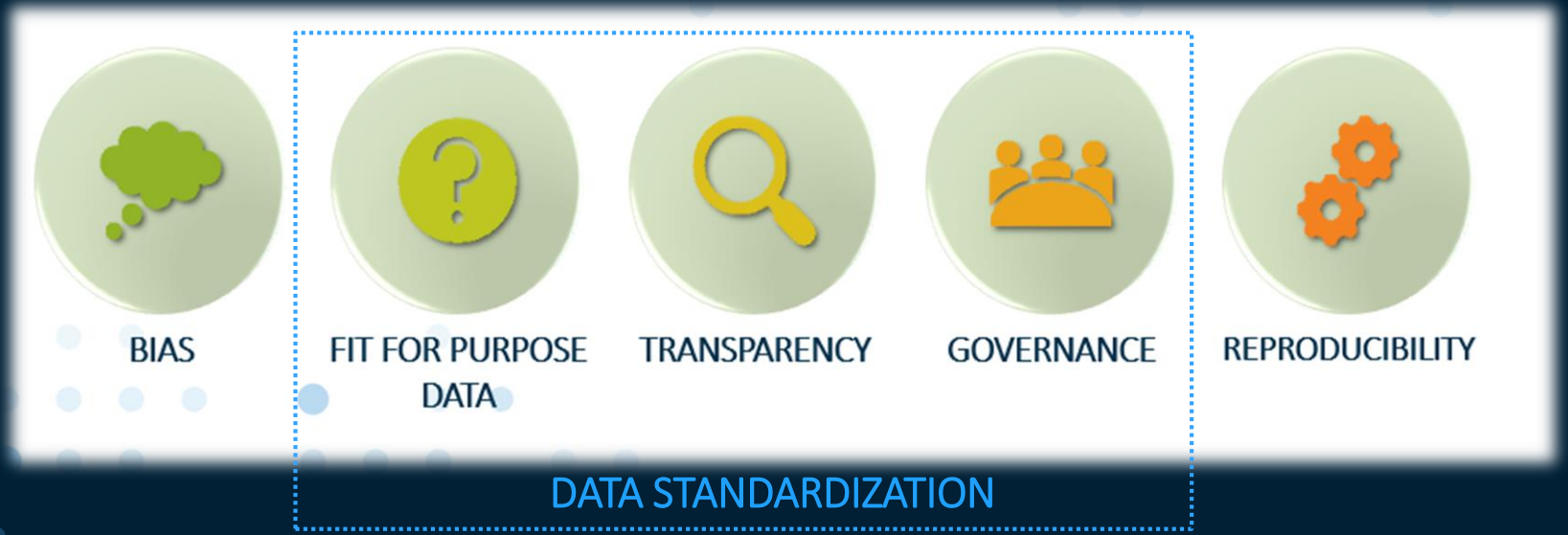
Linked In Post

cdisc eu interchange 2021 (E3C article)

Real World Evidence

How does SAS support our customers ?

Overcome challenges



SAS® Health

Data Management

Exploratory Analyses

Accelerated Analytics and Reporting

SAS® Health

SAS® Health Solutions

SAS® Life Science Analytics Framework
SAS® Cohort Builder
SAS® Episode Builder
SAS® Data Mapper
SAS® Visual Investigator

SAS® Health Analytic Insight Modules - AIMs

Payer
Provider
Public Health
Life Sciences

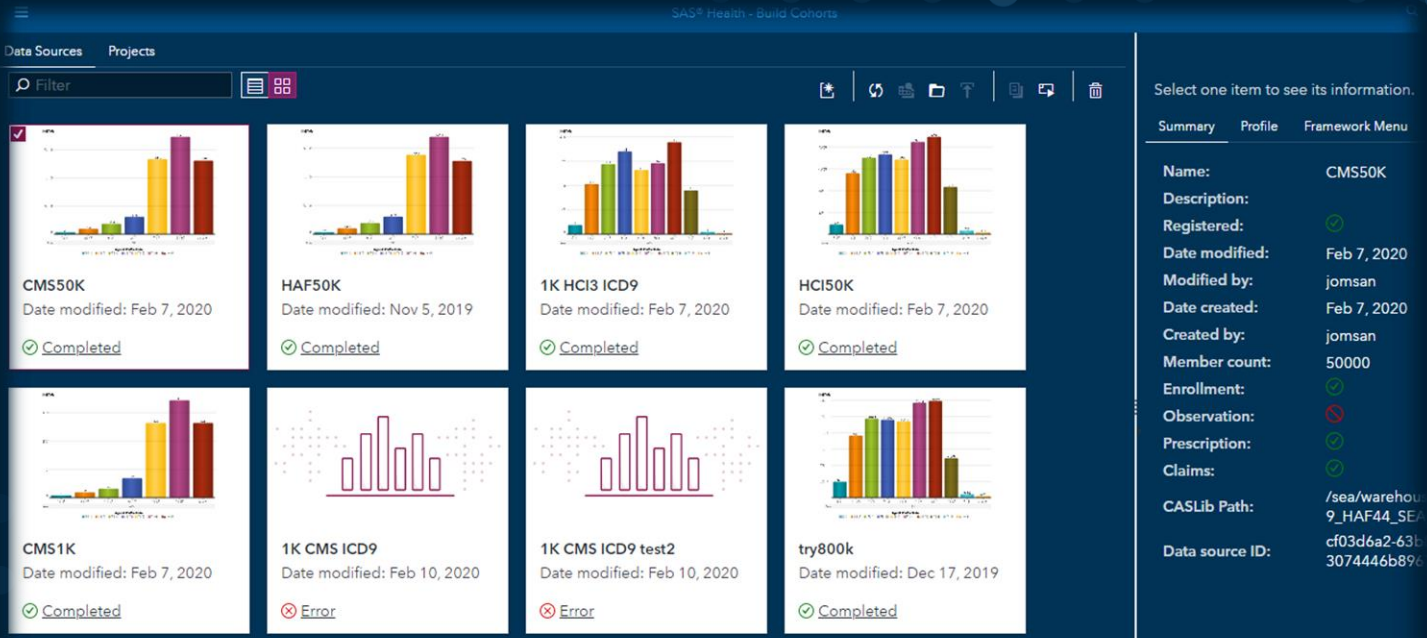
(App Store)

SAS® Analytics

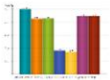
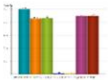
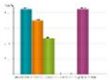
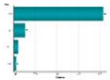
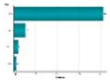
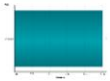
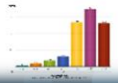
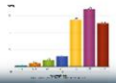
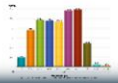
Manage the complete Analytical Life Cycle for Continuous Innovation

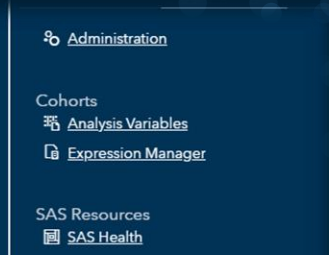
https://www.sas.com/en_us/software/health.html

SAS® Health – Managing your Data Sources

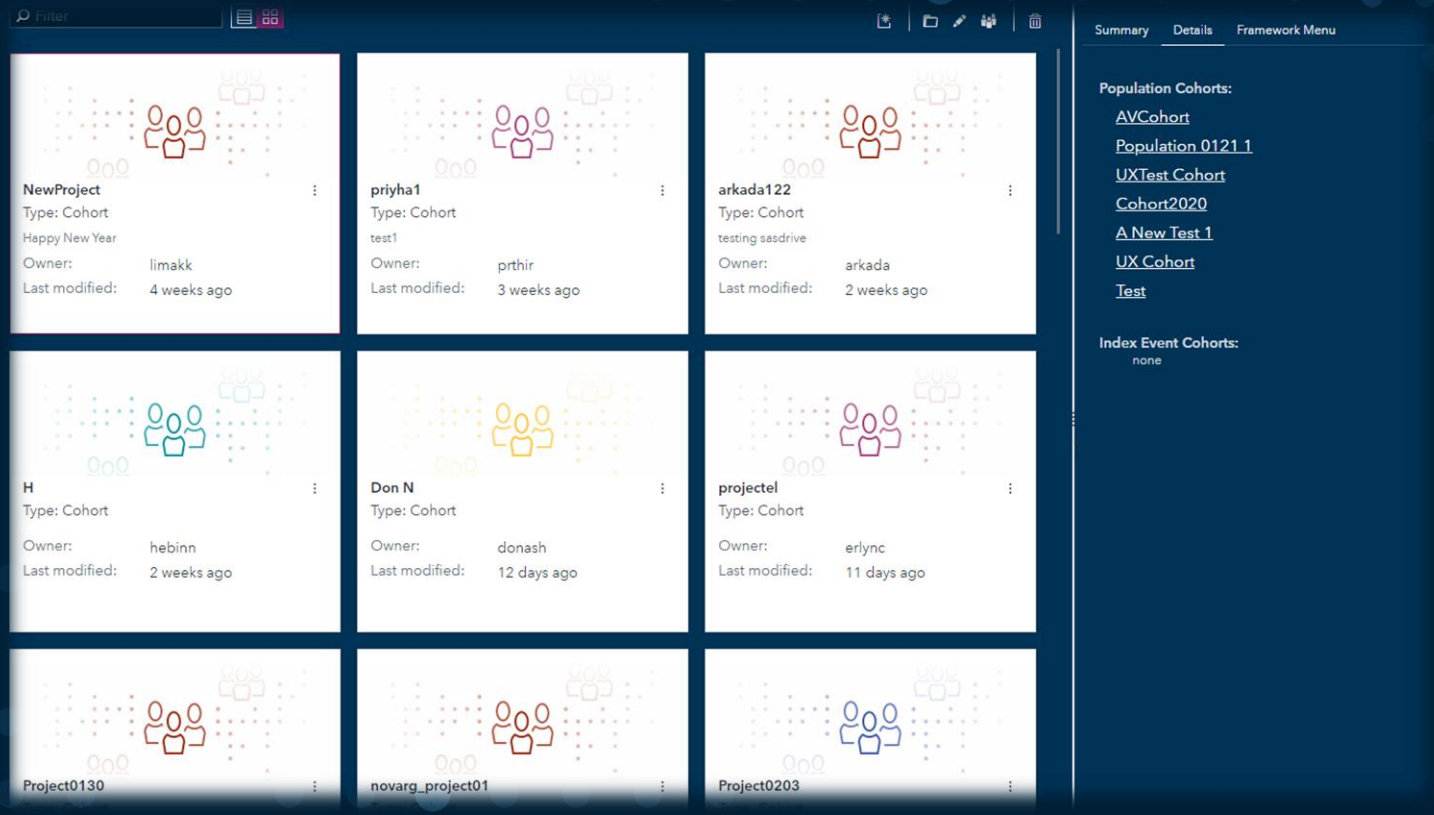


SAS® Health – Data Sources insights

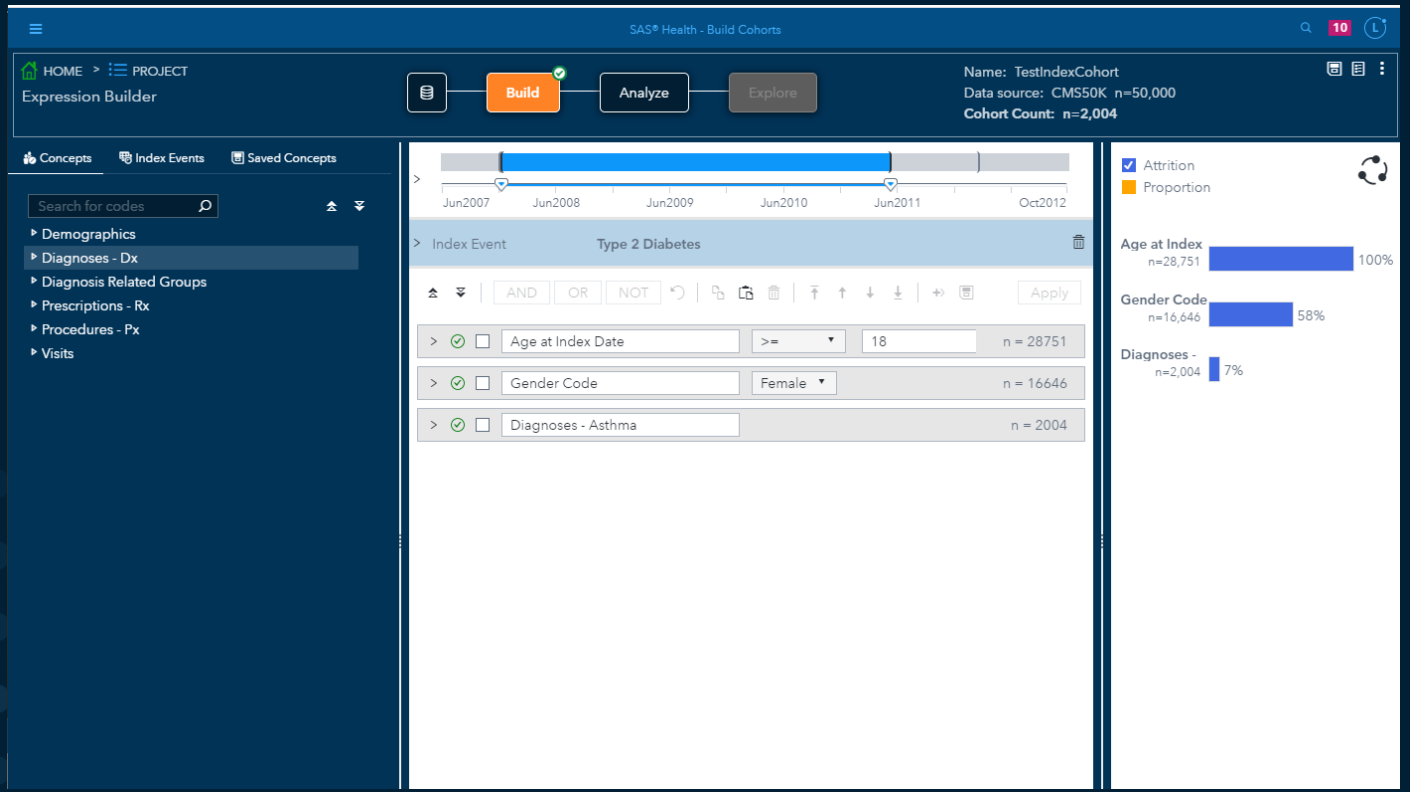
Data sources	CMS50K	HAF50K	try800k
Member count	50000	50000	843524
Enrollment	✓	✓	✓
Observation	⊘	✓	⊘
Prescription	✓	✓	✓
Claims	✓	✓	✓
Distribution Metrics			
Distribution By Gender			
Distribution By Ethinicity			
Distribution By Race			
Distribution By Age Group			



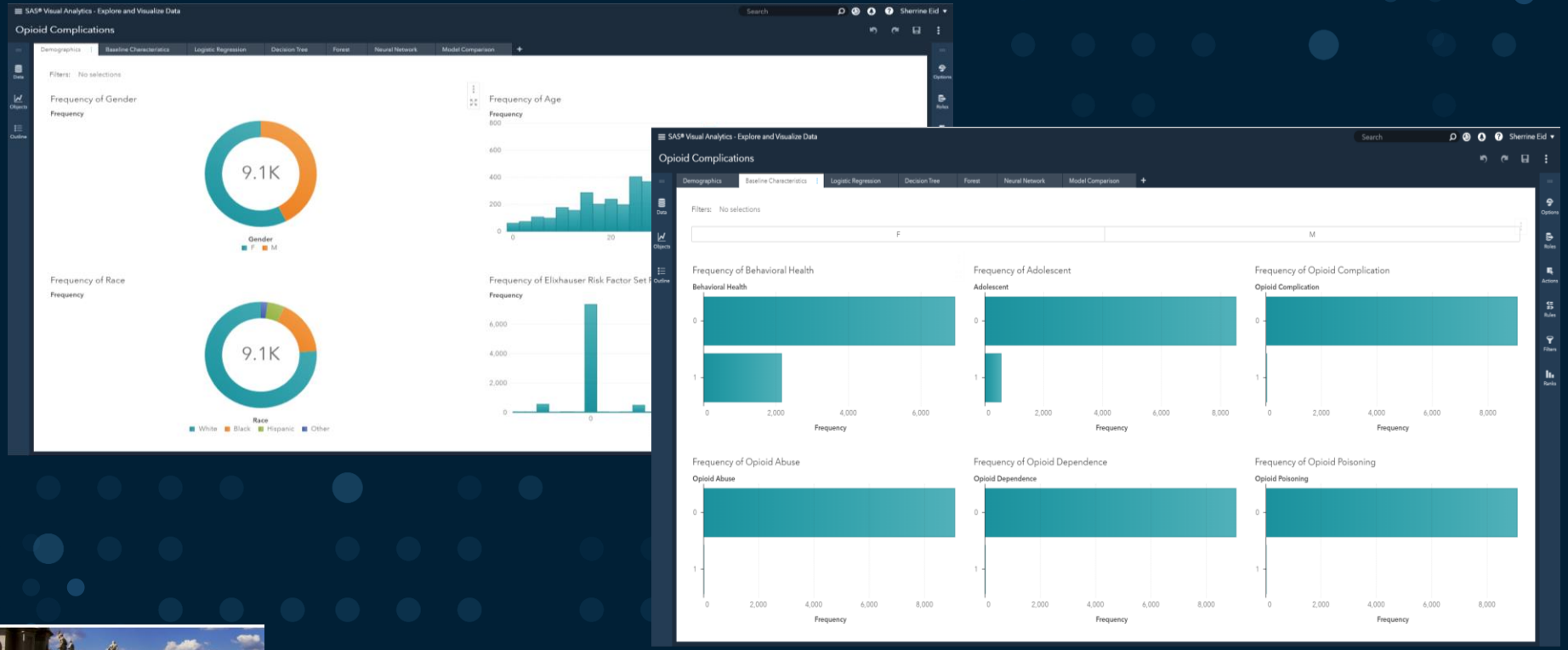
SAS® Health – Collaboration across departments and user Profiles



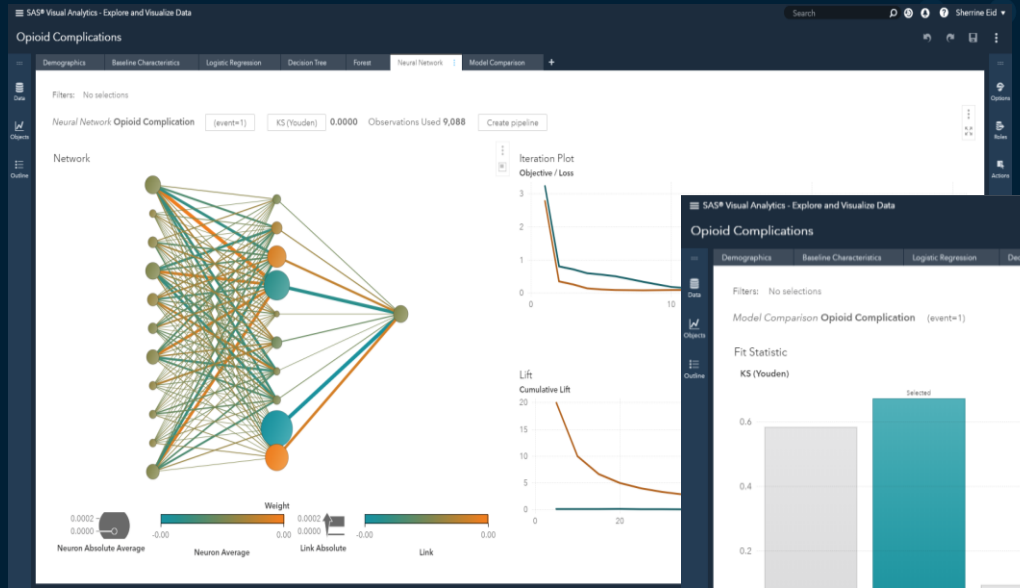
SAS® Health – Building Cohorts in minutes/hours instead of days/weeks

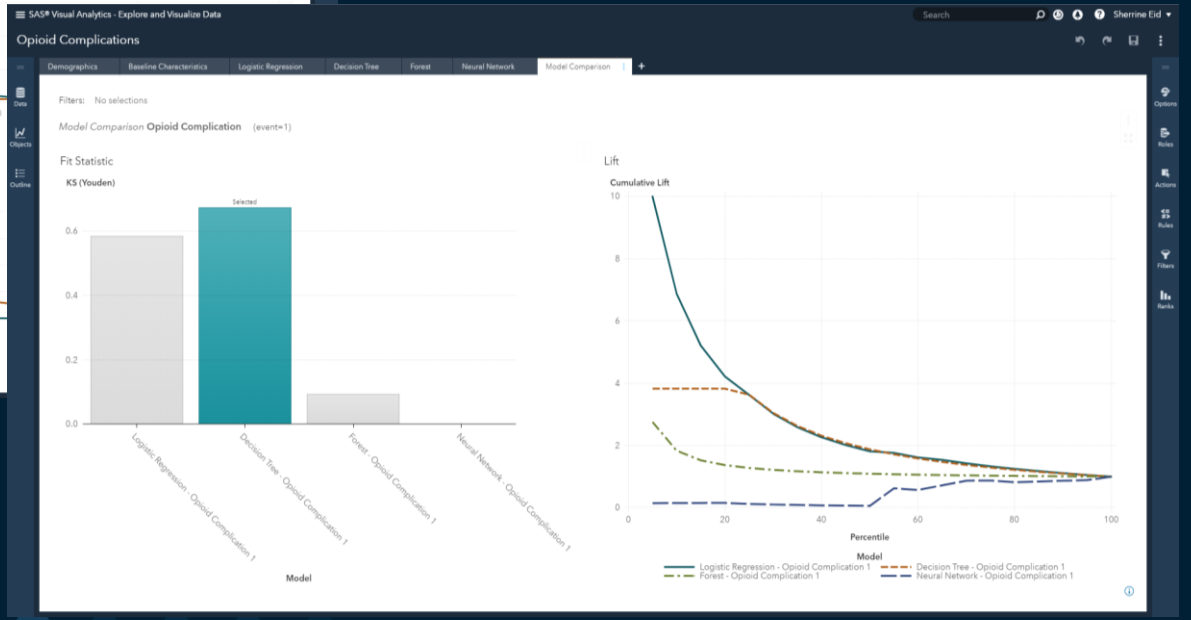


SAS® Health – Analysis on Cohorts of interest



SAS® Health – Analysis on Cohorts of interest





SAS® Health – Meet Regulatory Requirements

Submit Code and Logs

SAS® Studio

New Options View Open Save All

Tasks

Filter

- Optimization and Network Anal…
- Statistical Process Control
- SAS Viya Cloud Analytic Services
- SAS Viya Prepare and Explore …
- SAS Viya Evaluate and Implem…
- SAS Viya Statistics
- SAS Viya Machine Learning
  - Automated Machine Learning
  - Unsupervised Learning
  - Supervised Learning
    - Neural Network
    - Forest
    - Gradient Boosting
    - Factorization Machine
    - Support Vector Machine
    - Bayesian Network

Start Page | * New_Synthea_Prep.sas | * Gradient Boosting.ctk ×

Run Cancel Copy to My Tasks (0)

DATA OPTIONS OUTPUT INFORMATION

DATA

PUBLIC.SYNTHEA_100K_NTP…

Partition Data

Input data contains training data. Include:

Validation data

Test data

ROLES

Target

Use a nominal target

Task Console (0)

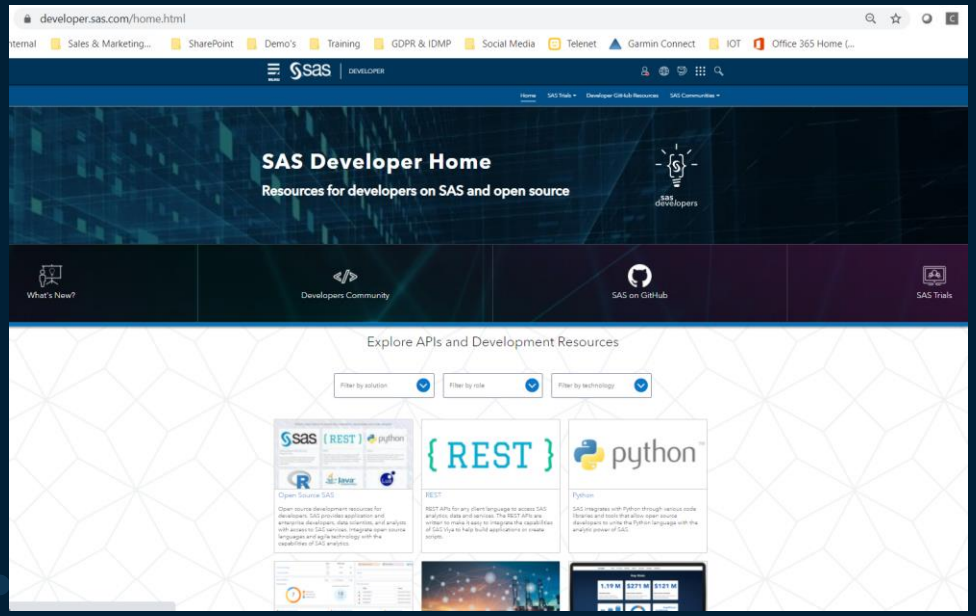
No items

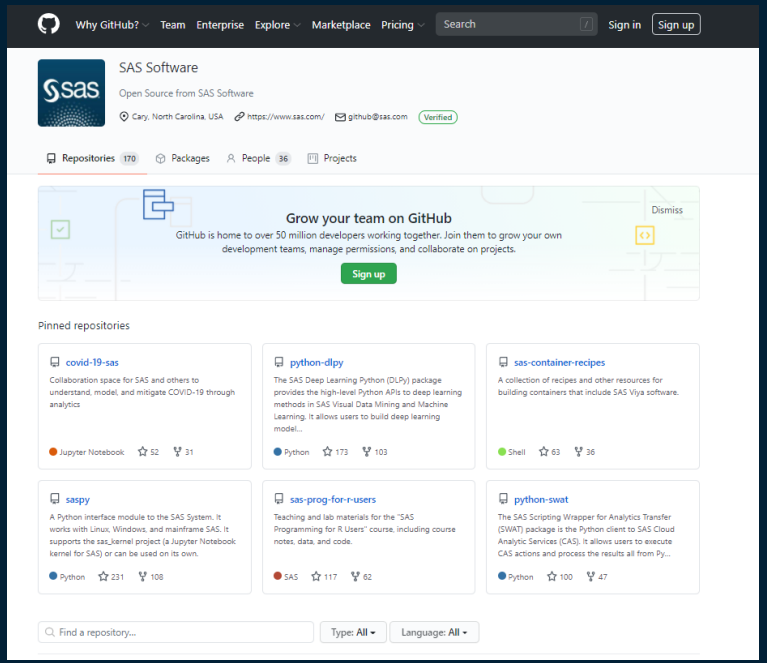
Code Log Edit

```
1  /*
2   *
3   * Task code generated by SAS® Studio 5.2
4   *
5   * Generated on '9/4/20, 4:18 PM'
6   * Generated by 'robcol'
7   * Generated on server 'CPOC-Viya-Services'
8   * Generated on SAS platform 'Linux LIN X64 3.10.0-957.5.1.e
9   * Generated on SAS version 'V.03.05M0P11112019'
10  * Generated on browser 'Mozilla/5.0 (Windows NT 10.0; Win64
11  * Generated on web client 'https://sashlslab.eastus.cloudap
12  */
13
14 ods noproctitle;
15
16 proc gradboost data=PUBLIC.SYNTHEA_100K_NTPROBNP;
17     target VALUE_N / level=nominal;
18     input DATE / level=interval;
19 run;
```

Gradient Boosting [temp] Line 1 Column 1 Recover (19) Submission (0)

SAS® Health – OPEN platform





Developer.SAS.com and SAS on GitHub

Any Questions ?

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Twitter @StijnRogiers

sas.com

Thank you !

sas.com