

# Development of a real-world evidence platform in NF2 to examine treatment patterns and outcomes



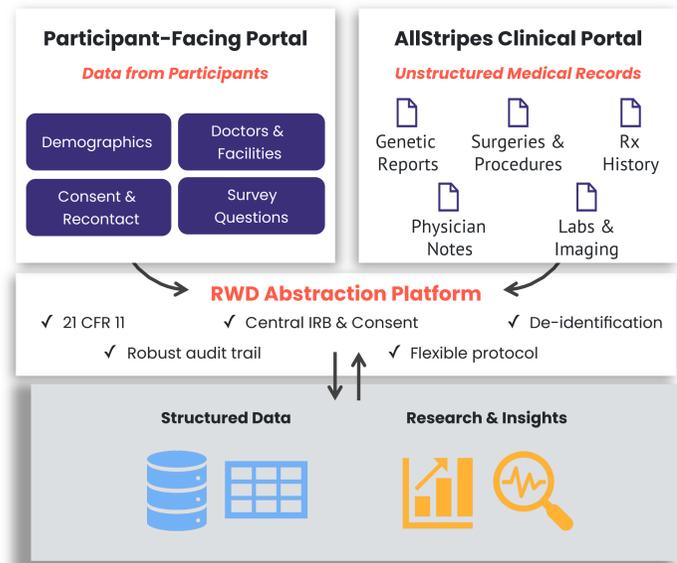
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## Introduction

NF2 is a rare disease, and its low prevalence can make it challenging to conduct clinical research on long-term clinical outcomes of patients who use off-label therapies for management. Real-world data (RWD), or data collected outside of clinical trials, can be used to understand disease progression, treatment patterns, and outcomes. Here, we describe the development of a RWD platform that allows for abstraction of high-quality data from medical records of patients with NF2.

## Methods

Figure 1: AllStripes RWD Platform Workflow



This work was performed in compliance with Western IRB. A broad, umbrella consent was developed to allow for de-identified data from medical records to be used in minimal risk research.

**Cohort:** Recruitment occurred via digital marketing and collaboration with the Children's Tumor Foundation and NF2 BioSolutions. Patients/guardians were asked to sign a HIPAA release, provide a list of hospitals and clinics where the patient receives care, and sign a consent form approved under a central IRB. Complete medical records were requested and digitized; re-requests were made every 6–12 months. Individuals were included in the study if clinical notes confirmed a diagnosis of NF2.

## Methods

**Clinical Data Abstraction:** In order to structure patient journeys, an iterative protocol consisting of clinical modules that define data to abstract from medical records was developed. When possible, modules were designed according to CDASH standards. Modules included demographics, diagnosis, surgeries and procedures, healthcare utilization (ER visits and hospitalizations), tumor history, and disease-related medications. During abstraction into the RDMD database, an electronic audit trail to the source documents was maintained for quality assurance.

**Data Analysis:** Structured, de-identified data was exported, and analysis was performed using descriptive statistics. Analysis was performed only on data abstracted from patients who had consented to research.

## Results

Figure 2: NF2 Cohort Demographics

36 patients consented to research and had medical records for review. The mean patient age was 34 years (SD±16.4); a majority of the cohort was female and from the West or South census regions of the United States.

| Patients               | N  | Female (%) | Male (%)   |
|------------------------|----|------------|------------|
| Consented with Records | 36 | 21 (58.3%) | 15 (41.7%) |

Geographic Distribution (N)



Current Age (Years)

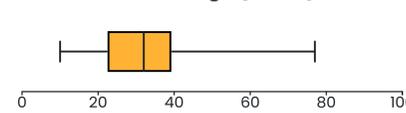


Figure 3: Medical Record Assessment

The majority of patients listed > 1 hospital or clinic. Medical records were requested from 5 years prior to self-reported date of diagnosis. All clinical documents were classified by specialty and note type; the most common document types available for review were radiology and neurology records.

| Document Statistics          | Median (Min–Max) |
|------------------------------|------------------|
| Medical Facilities Listed    | 4 (1–15)         |
| Clinical Documents Available | 93.5 (19–1634)   |
| Years of Data                | 10.1 (0.8–45.5)  |

## Results

Figure 3: NF2 Medical Record Assessment

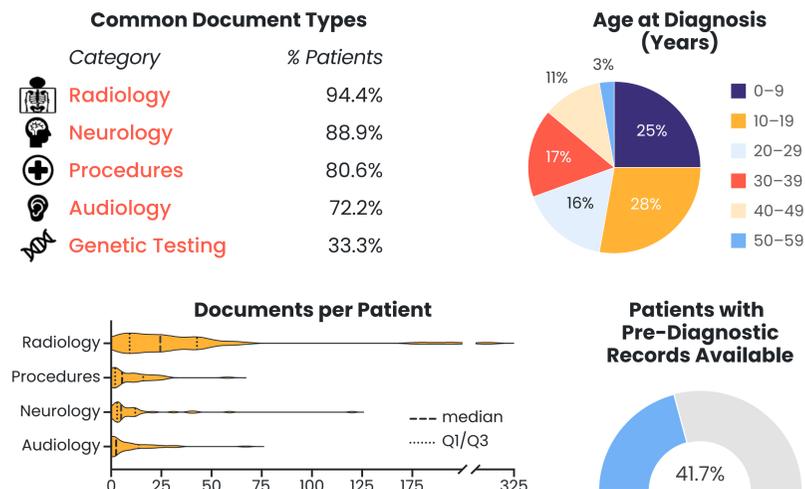
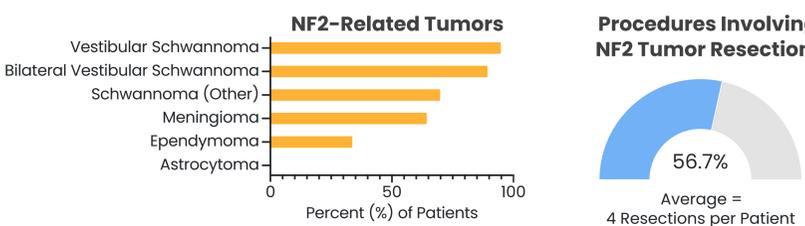


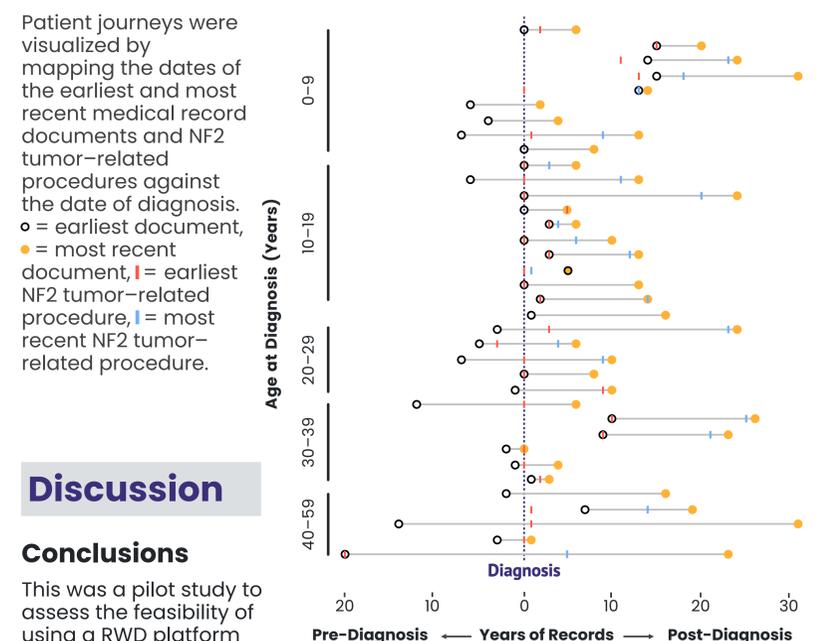
Figure 4: NF2-Related Tumors and Management

NF2-related tumors, medications, and procedures were abstracted from the medical records. All patients had at least one NF2-related tumor reported in their medical record; 86% had two or more. 33 patients (91.7%) had procedures recorded in their medical record (average = 6 procedures/patient); of these, 32 patients (88.9%) had NF2-related procedures. 12 patients had NF2-related medication data available for review; 10 total medications were identified.



## Results

Figure 5: NF2 Patient Journey Overview



## Discussion

### Conclusions

This was a pilot study to assess the feasibility of using a RWD platform to examine NF2 patient journeys and clinical outcomes. We demonstrated that our site-less model allows for collection and review of complete medical records regardless of patient geography. Using five clinical modules, we were able to glean insights regarding NF2 patient diagnosis, healthcare utilization, management, and tumor burden. Data abstracted from records can be used to construct individual patient journeys or to compare journeys across a cohort. Future work will focus on understanding NF2 patient management and outcomes across a larger sample size.

### Limitations

This was a small feasibility study and should not be considered a representative sample of all NF2 patients. Hospital lists are also provided by patients/guardians, and there may be unidentified gaps in care. Additionally, the results must be interpreted within the context of the limitations of RWD.

### Acknowledgements

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### References

1. Evans (2018). GeneReviews: Neurofibromatosis 2.

