RWD128

# A Retrospective Claims Analysis Characterizing Health Care Resource Use Among Patients with Friedreich Ataxia in the United States

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## 1. Background

- Friedreich Ataxia (FA or FRDA) is a progressive and systemic neurologic movement disorder, typically characterized by muscle weakness, worsening ataxia and scoliosis, resulting in loss of ambulation. Speech is impacted, and many patients experience loss of vision and hearing.[1]
- Cardiomyopathy and diabetes mellitus are common and serious manifestations of the disease.[1]
- Direct medical, non-medical, and indirect costs contribute to the overall economic burden of disease. Real world evidence could provide an understanding of the direct economic burden of FA that has not been well captured by other methods, such as patient and physician surveys.
- **This study aimed to characterize real-world health care resource use (HCRU) among commercially insured patients with FA in the United States (US) and compare that to non-FA patients.**

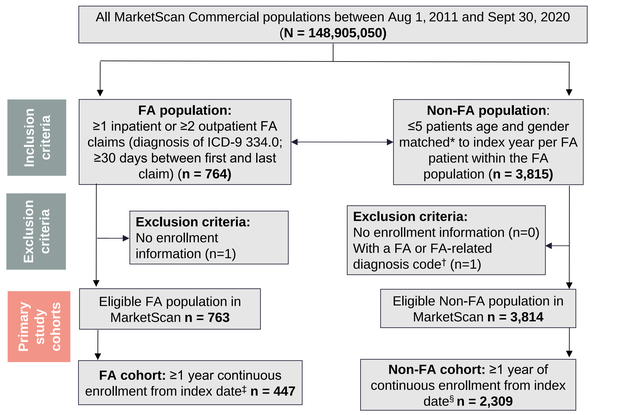
## 2. Methods

**Data source:** US Merative MarketScan Commercial database (Aug 2010 to Sept 2020)

**Inclusion (Figure 1):** Children and adults with ≥12 months of follow-up and ≥1 inpatient, or ≥2 outpatient visits separated by ≥30 days, with primary or secondary diagnosis of FA (ICD-9 334.0) prior to Oct 2015 (334.0 was replaced by a non-specific ICD-10 code in September 2015)

**Analysis:** HCRU by service type, age, and select clinical features were summarized and compared to a 5:1 age-, sex-, and index-year-matched comparison non-FA cohort. The presence of loss of ambulation, diabetes, scoliosis, and cardiomyopathy were used as proxies for disease severity to summarize HCRU by the number of disease severity indicators

**Figure 1. Cohort selection: inclusion/ exclusion criteria**




** Matching performed by data provider; † ICD-9 334.0, 334.3; ICD-10 G11.11, G11.1, G11.9, G11.18, G11.2, or G71.0; ‡Defined as date of first observed FA claim §Defined as the date of a randomly selected claim in the patient's claim history between August 1, 2010 and September 30, 2015.*

## 3. Results

### Cohort characteristics

- 447 patients with FA (mean [standard deviation, SD] age of 34.9 [17.5] years at index) and 2,309 non-FA comparison patients (35.8 [17.5]) years) met study criteria **(Table 1)**.

**Table 1. FA and non-FA cohort characteristics**

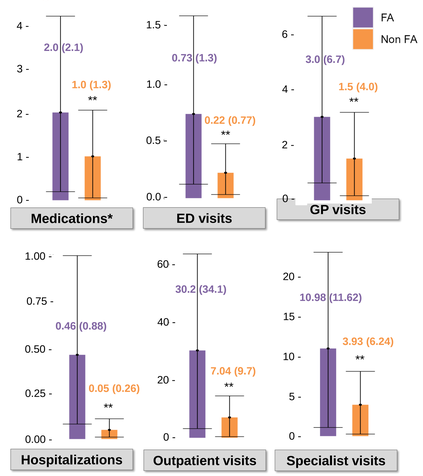
| | FA Cohort (N=447) | Non-FA Cohort (N=2,309) |
|---|---|---|
| **Total follow-up*, months, mean (SD)** | 42.79 (23.15) | 34.55 (23.15) |
| **Age, mean (SD)** | 34.93 (17.48) | 35.79 (17.48) |
| **Age categories*, n (%)** | | |
| 0-6 | 10 ( 2.24%) | 41 ( 1.78%) |
| 7-14 | 47 (10.51%) | 249 (10.78%) |
| 15-23 | 76 (17.00%) | 423 (18.32%) |
| 24-30 | 70 (15.66%) | 291 (12.60%) |
| 31-50 | 131 (29.31%) | 679 (29.41%) |
| 51-64 | 113 (25.28%) | 626 (27.11%) |
| **Sex, n (%)** | | |
| Male | 210 (46.98%) | 1072 (46.43%) |
| Female | 237 (53.02%) | 1237 (53.57%) |
| **Region, n (%)** | | |
| Northeast | 80 (17.90%) | 431 (18.67%) |
| North central | 94 (21.03%) | 466 (20.18%) |
| South | 175 (39.15%) | 897 (38.85%) |
| West | 88 (19.69%) | 475 (20.57%) |
| Unknown | 10 ( 2.24%) | 40 ( 1.73%) |

**Assessed at index date, defined as the date of the first recorded FA claim. In instances where patients have a single outpatient FA claim in their claim history that precedes the date of ≥1 inpatient FA claims, the date of the first inpatient FA claim will be used. Index date is included in follow-up period.*
*FA, Friedreich ataxia; SD, standard deviation*

### HCRU

- Having adjusted for the effects of age, sex, and index-year through matching, HCRU was significantly higher among those with FA across all resource types (all p<0.001), with the most frequent being outpatient visits **(Figure 2)**.
- FA patients had an annual mean (SD) of 30.2 (34.1) outpatient visits per patient compared to 7.0 (9.7) without FA.
- Among the outpatient visits in the FA cohort, 6.8 (15.5) annual visits were to physiotherapists, 3.1 (6.7) to general practitioners, 1.2 (3.2) to neurologists, and 1.0 (1.9) to cardiologists.
- Across categories of specialist visits, patients with FA also had statistically significantly higher numbers of visits than in the non-FA cohort, with the exception of psychiatrist visits.

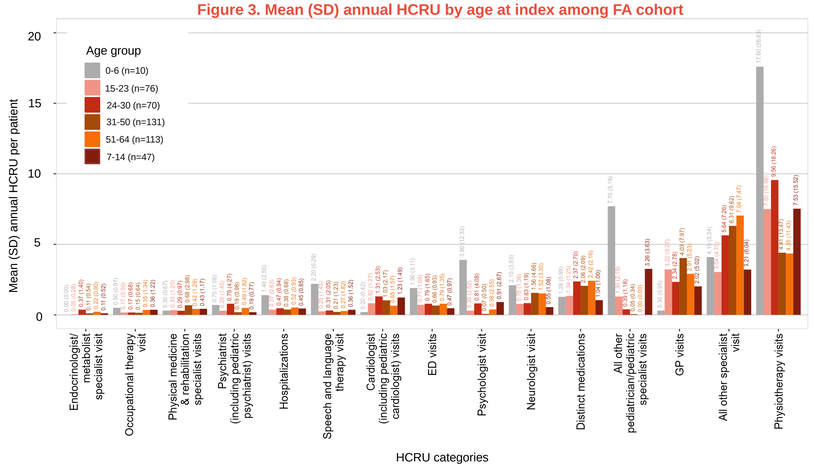
**Figure 2. Mean (SD) annual all-cause HCRU per person**




*Pharmaceutically equivalent products with the same dispensing form and strength
** p<0.0001; Two-proportion z-test was used to calculate statistical significance for the discrete proportions, and t-test was performed to compare the mean number of resource use between cohort groups as continuous, non-integer counts (averaged over each month)
Abbreviations: ED, emergency department; FA, Friedreich ataxia; GP, general practitioner; HCRU, health care resource use; SD, standard deviation

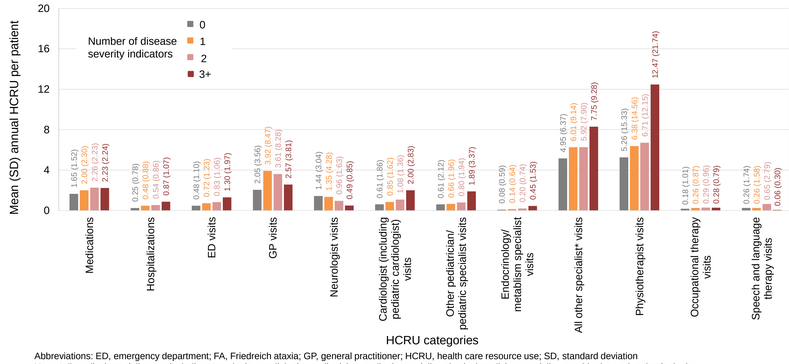
- Significantly more FA patients had ≥1 annual hospitalization or emergency department (ED) visit compared to non-FA patients (hospitalization: 30% vs. 5%; ED: 41% vs. 15%, p<0.001).
- When stratified by age at index, HCRU generally increased with age, with the highest used HCRU being physiotherapy and specialist visits **(Figure 3)**.
- Corrective procedures were not observed for all patients with diagnostic code for scoliosis (most surgery occurred at age of 13-14 years).
- Most categories of HCRU increase with number of FA disease severity indicator counts, with the exception of GP and neurology visits **(Figure 4)**.

**Figure 3. Mean (SD) annual HCRU by age at index among FA cohort**




Abbreviations: ED, emergency department; FA, Friedreich ataxia; GP, general practitioner; HCRU, health care resource use; SD, standard deviation

**Figure 4. Mean (SD) annual HCRU by number of disease severity indicators among FA cohort**




Abbreviations: ED, emergency department; FA, Friedreich ataxia; GP, general practitioner; HCRU, health care resource use; SD, standard deviation
Note: *all medical specialists not including neurologist, cardiologist, pediatrician, pediatric specialist, physical medicine specialist, psychiatrist, and endocrinologist

## 4. DISCUSSION AND CONCLUSIONS

- Patients with FA have significantly higher rates of HCRU, when compared to non-FA.
- The age distribution of the FA cohort suggests potential over-representation of patients with later-onset disease, (associated with lower disease severity and slower progression vs. early onset), therefore, the results should be interpreted in this context, knowing that the early onset, most severe patients with FA may have been under-represented.
- Nonetheless, this study reinforces the multidisciplinary care required for this complex disease, with high frequency of outpatient visits across many different specialties.
- The challenges of coordinating care across such a multidisciplinary team for the patients, caregivers, and health care system should not be underestimated.
- Currently, there is a need for disease modifying treatment options for FA; as such, findings from this study can help better estimate the impact of new interventions on the health care system.

**References**

1. Cook A & Giunti. British medical bulletin. 2017:1-12

**Disclosures**

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