Investigating IBM and VCP Patient Characteristics in Rural and Urban areas using the AllStripes Research Real-World Data Platform

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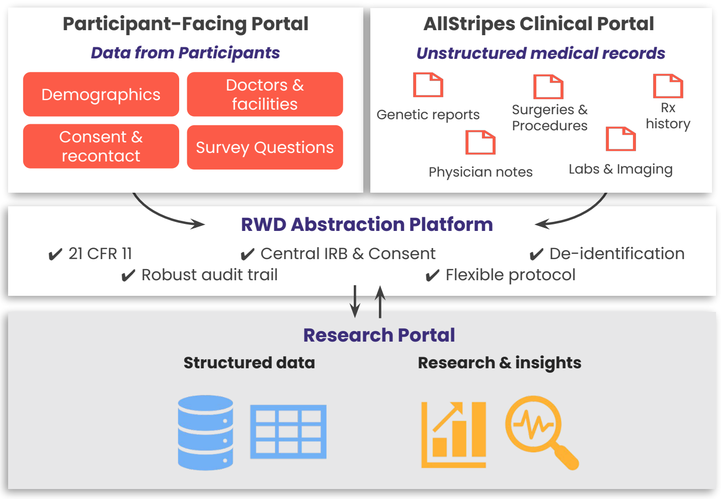
Introduction

Patients with rare diseases face multiple barriers to care, including availability of developed therapies and distance to care for clinical specialists and clinical trials. Real-world data (RWD), i.e., data collected outside of clinical trial research, can be used to understand patient population characteristics and geographic distributions. RWD may help enable equitable care and provide research opportunities. We used a RWD platform to examine key demographic and geographic characteristics of patients from two rare indications: inclusion body myositis (IBM) and mutations of the valosin-containing protein gene known as VCP. IBM is a rare condition that causes muscle weakness and degeneration in certain areas of the body. VCP disease, which primarily manifests as Inclusion Body Myopathy associated with Paget's disease of bone and Frontotemporal Dementia (IBMPFD), is a genetic condition that can affect the muscles, bones, brain, and nerves.

Here, we demonstrate that a rare disease RWD platform can be to investigate the distribution of patients in the United States across rural and urban areas.

Methods

Figure 1: AllStripes RWD Platform Workflow



This work was performed in compliance with WIRB-Copernicus Group (WCG® 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 for IBM occurred via Myositis Support and Understanding; patients and families with VCP disease were recruited via the Cure VCP Disease, Inc. Patients were asked to complete the consent and HIPAA release form, provide a list of hospitals and clinics where they receive care, and answer surveys tied to demographic information. Complete medical records were requested and digitized; re-requests were made every 6-12 months. Individuals with a self-reported diagnosis of IBM or VCP disease were included in this cohort.

Rural - Urban Designation: A RWD platform, developed by AllStripes Research, was designed to abstract data from medical records of patients with rare diseases. Medical records from patient-reported institutions were collected and digitized. Research consent from patients enabled the abstraction of deidentified patient characteristic and clinical data from medical records. IBM and VCP patient demographic characteristics and zip codes were exported. Rural and urban designations were made based upon patient zipcodes. To do this, the USDA Economic Research Services' rural-urban commuting area (RUCA) code classifications (which are based upon U.S. census tracts) were applied to corresponding zipcode areas.

Data Analysis: All statistical analyses were performed using R version 4.0.5.

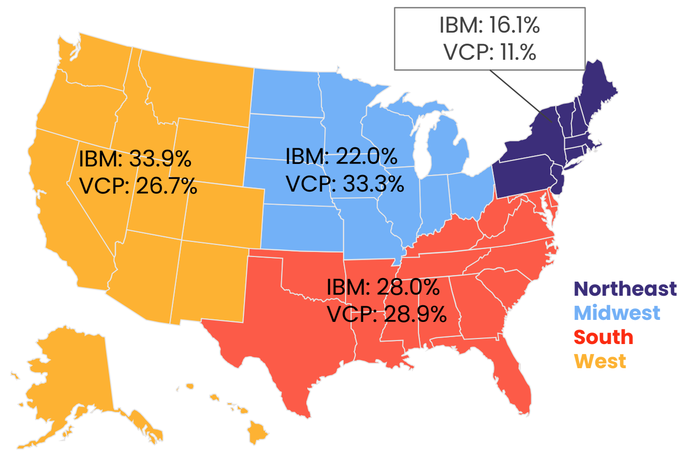
Results

One hundred eighteen IBM patients and 45 VCP patients had consented to provide data to the AllStripes Research platform. Among IBM patients, 66% were male and 34% female, and 45% of VCP patients were male and 55% female. The median age at diagnosis was 61 years for IBM (range 21-85) and 51 years for VCP (range 23-72).

Table 1: IBM and VCP Cohort Demographics

	IBM	VCP
Consented	118	45
Age at Diagnosis (years)		
Mean (SD)	59.2 (10.4)	49.1 (10.9)
Median [Min, Max]	61.0 [21.0, 85.0]	51.0 [23.0, 72.0]
Sex		
Female	36 (34.3%)	21 (55.3%)
Male	69 (65.7%)	17 (44.7%)
Region		
Midwest	26 (22.0%)	15 (33.3%)
Northeast	19 (16.1%)	5 (11.1%)
South	33 (28.0%)	13 (28.9%)
West	40 (33.9%)	12 (26.7%)
Population Density		
Rural	19 (16.1%)	9 (20.0%)
Urban	99 (83.9%)	36 (80.0%)

Figure 2: IBM and VCP Geographic Distribution



Note: Geographic distribution is based upon U.S. Census regions

Across the U.S., 16, 28, 22, and 34% of IBM patients reside in the Northeast, South, Midwest, and West regions of the United States, respectively; 11, 29, 33, and 27% of VCP patients reside in the Northeast, South, Midwest, and West regions, respectively. The majority of IBM and VCP patients resided in urban areas. Of the IBM patients, only 16% (n=19) live in rural areas. Similarly, of the VCP patients, only 20% (n=9) live in a rural area. This is similar to the national level among all U.S. residents where 19.3% live in rural areas. Among IBM patients, urban residence was associated with younger age at diagnosis (p=0.0329), but this was not observed among VCP patients (p=0.9156).

Table 2: Patient Characteristics Stratified by Rural/ Urban Status

	IBM		VCP	
	Rural	**Urban**	**Rural**	**Urban**
Consented	19	99	9	36
Age at Diagnosis (years)				
Mean (SD)	63.2 (6.3)	58.4 (10.9)	44.6 (14.4)	50.2 (9.7)
Median [Min, Max]	64.0 [52.0, 78.0]	60.0 [21.0, 85.0]	39.0 [23.0, 72.0]	52.5 [34.0, 71.0]
Sex				
Female	5 (31.3%)	31 (34.8%)	<5	18 (58.1%)
Male	11 (68.8%)	58 (65.2%)	<5	13 (41.9%)

Conclusions

To our knowledge this is the first study to investigate the distribution of IBM and VCP patients in the United States across rural and urban areas. Most patients lived in urban areas and are distributed proportionally and similarly to the general United States population. While the majority of patients live in urban areas, most rare disease patients tend to live long distances from clinical trial sites, as has been shown in prior research. This suggests that opportunities to participate in clinical trials may be limited for rare disease patients. RWD offers the opportunity to identify patient geography and care patterns to aid in understanding how to address rare disease patient care.

Future work will use the AllStripes RWD platform to examine access to care and research across additional rare diseases in our research portfolio. As our platform also allows for abstraction and structuring of data from medical records to inform natural history, treatment outcomes, and healthcare utilization studies, future work will also examine other factors related to time of diagnosis.

Limitations

Due to the nature of IBM and VCP, this study was limited to a small sample size. Convenience sampling method was used via patients signing up on the AllStripes Research platform and should not be considered a representative sample of all IBM and VCP patients. This study assumes that patients location at the time of sign up is the same as their location at time of diagnosis.

Acknowledgements

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References

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