monARC
BIONETWORKS

Empowered Patients, Faster Cures

Komathi Stem
Founder and CEO
Current State: Patients are Passive Consumers

- Clean
- Structured
- Limited
- Expensive
- Time Consuming
- Proprietary
THREE BIG TRENDS
Point of Care is Shifting

95% - cellphone
77% - smartphone
20% - online access

75% - Physicians
75% - Willing to use

Source: 2018 Pew Research
2018 Physician Practice Survey
Data is abundant but Siloed & Noisy

83% - EHR Adoption

Variety of Data beyond the HER

Interoperability

Security
Data Access is More Important than Ownership

Access
Integrate
Mine
New Players
- Amazon
- Apple
Big Opportunity: Patients as Partners
Patients are Ideal Aggregators of RWD

- Own the data
- Access to data
- 96% - willing to share
- Security
- Trust
Patients will Generate RWI to inform new Endpoints

Meaningful Endpoints

Influence Design

Decision Making
Continuous RWD Trial => Dynamic Label

Direct to Patient

Personalization of data to inform decision making at the point of care will be an expectation.
moARC’s Integrated RWD Learning Platform

- Data collection
- Machine Learning/AI Analytics

Virtual Research Network
Natural history Study

Insights for trial design
Trial Ready Patients

Adaptive Pragmatic
Connected Clinical trial
CASE STUDY
IRB Approval to Published Poster in 4 months

AN OBSERVATIONAL STUDY TO BETTER UNDERSTAND THE ADHERENCE AND USE OF HOME-BASED DIGITAL DEVICES TO MEASURE DISEASE-RELEVANT OUTCOMES IN PATIENTS WITH IDIOPATHIC PULMONARY FIBROSIS AND TO ASSESS FEASIBILITY FOR FUTURE STUDIES

Switzer, T.1, Stem, K.3, Nagra, S.3,., Adams, H.2, and Belloni, P.1
1Genentech Inc., South San Francisco, CA; 2Roche, Basel, Switzerland; and 3monARC Bionetworks, Inc., Palo Alto, CA

BACKGROUND & INTRODUCTION
• Idiopathic Pulmonary Fibrosis (IPF) is a disease with unpredictable progression rates and debilitating symptoms that include fatigue, breathlessness, and dyspnea.
• Estimated median survival is 3-5 years from diagnosis.
• Assessment of IPF progression is typically performed within the clinic and clinic visits are not frequent enough to capture progression in real time.
• Measuring disease progression and treatment effect along with assessing the impact of disease on patient’s daily life is currently limited to instruments that are used in the clinic.
• Daily symptom diary has been used, but rely on patient recall and are limited to data collection on specific times and days.
• To date, home-based digital devices have been utilized in single center studies assessing the use of portable spirometry to detect disease progression associated with IPF but not in clinical trial research.
• Digital patient friendly tools have the potential to reduce patient time and travel burden to clinics while collecting more continuous data into the progression and management of IPF in the home setting.
• Data on the usability, scalability, and efficacy of home-based digital devices to collect disease and treatment outcomes will enhance evidence for the broad application of such tools.
• This study GA3830 was a feasibility study to evaluate the use and adherence of home-based digital devices (spiroometric wearable and sleep monitor watch, wireless body weight scale, and an IPF symptom diary app) to collect clinically relevant data in IPF patients.

Objectives
Primary Objective
• To evaluate the feasibility of using digital devices to collect clinically relevant data in idiopathic Pulmonary Fibrosis (IPF) patients in the home.

Secondary Objectives
• To assess patient usability and adherence of the wearable spirometer, wearable activity and sleep data using a wearable activity tracker and body weight using the body weight scale.
• To assess patient diary and treatment symptoms on daily diary app.
• To qualitatively assess patient spirometer device preference.

METHODS
• Patients with IPF (n=21, 49.5% male, median age 65.5 yrs with range of 55-81 yrs) were randomized into baseline phase of the study to receive either the home-based IPF device system (PAH carecare device (PDA); SpierCare (SCA); Porta-Therapy (PTT)) or the Medical International Research SpierCare (MIR-PTT).
• On Day 14, patients switched from the first spirometer to the second spirometer and the monitoring was continued for an additional 14 days.
• At the end of the study, patients will rate their spirometer device preference and provide reasons for their preference.
• Subjective feedback data was collected through 3 phone interviews with each of the participants. Interviews were conducted on Day 2 after baseline visit and training on devices first spirometer, Day 15 (after user assessment and training on second spirometer), and Day 28 (the last visit).
• On Day 28, each subject provided subjective feedback relating their experiences with both spirometer devices and detailed feedback regarding use of the watch, weight scale, and IPF app.

RESULTS

Within Patient In-Home Spirometry Reproducibility

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<th>Measurement</th>
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Historical In-Clinic vs In-Home Spirometry

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Patient Adherence

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Subjective Outcomes:

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IPF Symptoms vs Steps per Day

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CONCLUSIONS

• High Patient adherence rates demonstrates that it is feasible and reliable to use digital tools for home-based collection of clinical relevant data from patients with IPF over a 30 day period.
• Relative reproducibility of repeated FVC measurements within a single IPF patient using a home spirometer was similar across both spirometers and similar coefficient of variation was reported by process studies in daily home-based spirometry for health subjects, COPD patients, and IPF patients.
• Home Spirometry underestimated the FVC value compared to historical in-clinic FVC measurements. This decrease could be due to different test or disease progression.
• Subjective interviews with patients indicated that patients preferred to blow less frequently. Heat when data was shared with them and several enjoyed tracking their data using a mobile application.
• For a few patients sleeping with the watch was difficult which makes it a difficult form factor to use for long periods of time.
• Daily step count using the watch was feasible and showed possible correlation to IPF symptoms. However, a longer sample size and longer observation period is needed.

REFERENCES

ACKNOWLEDGEMENTS
We would like to thank all the patients who devoted their times and data to this research study.
Big Change Requires Incentives
Next Generation Drug Development Needs

Incentives for

- Data Sharing across researchers/sponsors
- Novel Endpoint Development

Improved Legislation to broaden access state borders

- State laws are limiting virtual trial capabilities
- Investigational Drug Shipment across borders

New Capabilities with non-traditional partners

Social networking, mobile devices, user experience, IOT, AI, security and cloud infrastructure for big data storage and analytics

Bold Partnerships with Patients
Thank you

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