Health Disparities in the MS Community

Track 2
Clarifying the Confusing World of Clinical Trials in Underserved Populations

Provided by MSAA
Multiple Sclerosis Association of America

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From Research to Therapy: 
Clarifying the Confusing World of Clinical Trials

Jacqueline Faulkner Rosenthal, MD 
Multiple Sclerosis Institute at Shepherd Center
Objectives

• Importance of diversity in research participation
• The latest research developments in MS treatment pertaining to ethnic minority groups
• The role research plays in providing evidence for treatment and quality-of-life decisions
• An understanding of clinical trials and what participation entails
• Overview of regulations in place to provide protection to research participants
• How to assess the benefits and risks of participation
Why Are Clinical Trials Important?

Evidence-based medicine

Tells us information on the effectiveness of various treatments
1. Geography, ethnicity, and race contribute to disease susceptibility.

2. The natural history and response to treatment may differ between different ethnic populations
   • What genetic factors influence disease?
   • Do all racial/ethnic groups respond similarly to treatment?

3. Socioeconomic status and access to care may lead to over-estimates in disease severity.
   • In order to accurately apply clinical trials results to ALL patients, we need to study ALL patients!
Clinical trials: <10% clinical trial participation from minorities

Why Are Minorities Underrepresented?

Mistrust

Access to care
- Insurance
- Income
- Language barriers

Cultural/religious beliefs
Minorities with MS

<1% of the MS literature has a focus on Black or Hispanic/Latino patient population

Very limited data on MS health outcomes in Hispanic Americans

MS in Minorities: What are we learning?
Minorities May Have Different Incidence Rates of Multiple Sclerosis

- Two studies comparing the incidence rate among different races/ethnicities have found that African Americans have a higher incidence rate than Caucasian Americans while Hispanic and Asian Americans have a lower risk of MS.

Differences in MS Age of Onset

African Americans may have an older or younger age at onset

Hispanic/Latino tend to have younger age at onset

• African Americans may have more aggressive/rapid disease course, more frequent relapse, poorer recovery after relapse, faster transition to secondary progressive MS

• African and Hispanic Americans may have more disability (i.e., walking impairment) accumulation
Hispanic Americans may have higher frequency of optic neuritis and transverse myelitis at onset

Opticospinal MS

• A variant of MS with selective involvement of the optic nerves and spinal cord

• More common among African Americans, Hispanic Americans, and Asian Americans
Differences in MRI Findings

African Americans may have higher T1/T2 lesion burdens

Accelerated Neurodegeneration?

• Compared to Caucasian Americans, African Americans may have faster brain and retinal tissue loss.

• This is in line with prior findings suggesting more severe disease course in African Americans.

The Genetics of MS Are Complex and Differ by Race/Ethnicity

• Differences in MS prevalence and clinical outcomes possibly explained, in part, by European ancestry

Differences in Response to Certain Treatments

• For example, interferons may not be as effective in African Americans

African Americans and Hispanics may not respond as well to higher serum vitamin D.

Vitamin D

Socioeconomic Barriers

- Limited insurance
- Access to specialty care
- Income
- Education levels
- Language and computer literacy

- African Americans less likely to have private insurance
- Hispanic Americans receive less mental health or rehabilitation care
- African Americans less likely to receive care at a MS clinic

Other Potential Racial/Ethnic Variances

Differences in mortality?

Differences in biomarkers?

Differences in immunopathogenesis?

- Immunopathogenesis = process of disease development as affected by the immune system.

Questions Needing to be Addressed

- Why are there differences in disease severity?
- What are the implications for treatment?
- What are the implications for accurate and timely diagnosis?
Clinical Trials

• A type of research study where individuals are assigned to one or more interventions to evaluate the effects of those interventions.

• Led by a principal investigator (PI)
# Types of Clinical Trials

<table>
<thead>
<tr>
<th>Types of Trials</th>
<th>Description</th>
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<tbody>
<tr>
<td>Prevention Trials</td>
<td>How can we prevent disease from occurring or returning</td>
</tr>
<tr>
<td>Screening Trials</td>
<td>Finding new ways to detect disease or certain health conditions</td>
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<tr>
<td>Diagnostic Trials</td>
<td>The examination and/or comparison of various tests or procedures used for diagnosing disease</td>
</tr>
<tr>
<td>Treatment Trials</td>
<td>New treatments, combinations of treatments, new surgeries or other therapies to treat disease</td>
</tr>
<tr>
<td>Behavioral Trials</td>
<td>The examination or comparison of behavioral changes that have been designed to improve health</td>
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<tr>
<td>Quality of Life Trials</td>
<td>How to improve the quality of life in individuals with certain medical conditions</td>
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</table>
Who Sponsors Clinical Trials?

- Pharmaceutical Companies
- Federal offices and agencies
  - National Institutes of Health
  - U.S. Department of Veterans Affairs
- Individuals
  - Doctors or health care providers
- Other organizations

Sponsors may initiate, manage, or finance the clinical trial, but do NOT actually conduct the research.
## Common Terminology

### Randomization
- The method by which treatments are assigned to each participant by chance, rather than by choice, to avoid bias.

### “Blinded” or “Masked”
- Prevents members of the research team from influencing the results.

### Intervention Group
- Drugs
- Devices
- Procedures
- Diet
- Therapy
- Exercise

### Placebo
- No treatment value

https://grants.nih.gov/policy/clinical-trials/definition.htm
## FDA Categories for Clinical Trials: 5 Phases

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<tr>
<td><strong>Phase 0</strong> Exploratory study involving very limited human exposure to the drug, with no therapeutic or diagnostic goals (for example, screening studies, microdose studies)</td>
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<tr>
<td><strong>Phase 1</strong> Researchers test an experimental drug or treatment in a small group of people (20 - 80) for the first time. The purpose is to learn about safety, determine a safe dosage range, and identify side effects.</td>
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<tr>
<td><strong>Phase 2</strong> The experimental drug or treatment is given to a larger group of people (100 - 300) to see if it is effective and to further evaluate its safety.</td>
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<tr>
<td><strong>Phase 3</strong> Large groups of people (1,000 – 3,000) to gather more information about safety and effectiveness by studying different populations and different dosages and by using the drug in combination with other drugs.</td>
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<tr>
<td><strong>Phase 4</strong> Researchers gather information about drug’s risks, benefits, and best use after it has been approved for use by the FDA and available to the public.</td>
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### Information Included in the Clinical Trial Protocol

<table>
<thead>
<tr>
<th>Study Goal</th>
<th>Eligibility</th>
<th>Protections against risks to participants</th>
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<tbody>
<tr>
<td></td>
<td>Specifics about tests, procedures, and treatments</td>
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<td></td>
<td>Study Duration</td>
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<td></td>
<td>What information will be gathered and analyzed</td>
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Clinical Trials Have Specific Guidelines: Eligibility Criteria

- Age
- Sex
- Type and Stage of Disease
- Previous Treatment History
- Other Medical Conditions
Expectations for Research Participants Vary

- Clinic Visits
- Procedures
- Medications
- Questionnaires
Protection for Research Participants

Government Agencies

• Office of Human Research Protections (OHRP)
• Food and Drug Administration (FDA)

Institutional Safeguards

• Institutional Review Boards (IRB)
• Data Safety Monitoring Boards (DSMBs)
Informed Consent: What is the Purpose?

- To ensure the research participant understands enough about the study to decide if they would like to participate
- When you sign the informed consent, you are indicating that you understand the research process and that you may leave at anytime
- Informed consent is NOT a contract
<table>
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<tr>
<th>Description</th>
<th>Expectation</th>
<th>Plan of Research or Protocol</th>
<th>Contact</th>
</tr>
</thead>
<tbody>
<tr>
<td>A description of the purpose of the research</td>
<td>What to expect as a participant</td>
<td>What is the plan of research or protocol</td>
<td>Who to contact with questions/concerns.</td>
</tr>
<tr>
<td>Possible risks or discomforts</td>
<td>Possible benefits</td>
<td></td>
<td></td>
</tr>
<tr>
<td>You can take the informed consent home to discuss with family/friends.</td>
<td>ASK QUESTIONS!</td>
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Addressing Misconceptions

- Informed consent is not a contract
  - You can leave the study at **ANY** time, for **ANY** reason.
- If you leave a study, it will **NOT** affect your care.
- Researchers **MUST** keep health information private.
- Placebo
  - Not all studies use a placebo
  - Placebos are not used if it means you would be put at risk by not having effective treatment.
  - If placebo is used in the study, you will be told **BEFORE** you participate in the trial
Questions to Ask

What is the research about?

What are the study requirements?

How might the research affect you?

Potential risks and how these will be minimized?

How will your health and personal information be protected?

Are there costs? Who will pay?

Who can you contact if you have questions/concerns?

Other considerations - Time/duration, travel, child-care
After the Clinical Trial

Researchers examine results and the significance of the results.

Results are typically published in a scientific journal.
Pros

• Access to otherwise unavailable treatments or procedures
• Contribute to the advancement of medical knowledge.
• Contribute to optimized disease management in minorities.
• Regular medical attention from a research team that includes doctors and other health professionals.

Cons

• Frequent visits
• Additional testing
• Possible risks
• You may not be part of the treatment/experimental group
Clinical Research: Alternatives to Clinical Trials

Study Design

Experimental Studies e.g. Controlled Clinical Trials

Observational Studies e.g. Patient Registries

Observation only; no intervention
Can provide useful information re: risk factors, safety, treatment, etc.
Take Home Points

• Volunteers are essential to clinical trials.
• You can benefit yourself and/or others.
• Research clearly suggests racial/ethnic and socioeconomic differences that need to be addressed in clinical trials.
• Adequate representation from minorities is critical.
Patient Resources

- Office of human research Protections (OHRP)

- To search for active research products by diagnosis and interest area
  - [https://clinicaltrials.gov/](https://clinicaltrials.gov/)

- FDA
  - [https://www.fda.gov/patients/clinical-trials-what-patients-need-know](https://www.fda.gov/patients/clinical-trials-what-patients-need-know)

- NIH
  - [https://newsinhealth.nih.gov/2015/03/be-partner-clinical-research](https://newsinhealth.nih.gov/2015/03/be-partner-clinical-research)
  - [https://www.nih.gov/health-information/nih-clinical-research-trials-you](https://www.nih.gov/health-information/nih-clinical-research-trials-you)
“BEAT-MS will enroll 156 adults ages 18 to 55 years at 19 sites in the United States and the United Kingdom. Participants will be randomly assigned to receive either AHSCT or one of the best available high-efficacy biologic drugs, and then will be followed for 6 years. The neurologists who periodically examine the participants and assess their level of disability will not know which type of treatment they were assigned.”
Questions?
Thank You

• This concludes our webinar: “From Research to Therapy: Clarifying the Confusing World of Clinical Trials.”

• MSAA would like to thank our funding partners Bristol Myers Squibb, EMD Serono, Genentech, and Sanofi Genzyme for supporting this series. We would also like to thank Dr. Jacqueline Faulkner Rosenthal for taking time out of her extremely busy schedule to provide us with this critically important information and Impact Education, LLC for their partnership in delivering this program.

• The next MSAA webinar will broadcast on Tuesday, June 30th at 8 pm Eastern with Dr. Mitzi Joi Williams and will conclude this series, Health Disparities in the MS Community series, Track 3: The African American Experience. Tonight’s webinar was recorded and will be archived to our website soon. Please visit MyMSAA.org for more details.

• Please consider completing a brief survey that immediately follows this slide. On behalf of MSAA, thank you for watching and stay safe!