



Consensus conference

Protocols

Data collection

Dissemination

Future Plans

ONLINE

READY TO GO!



Survey

Next steps

Friends of CARRA
JDM Practice Survey

Scenarios

N = 11



Team

Cases

What's been done?



Exit this survey >>

JDM Standards of Care Study: GROUP D SURVEY

A 5-year-old female presents with progressive muscle weakness and rash. Symptoms began 5 months ago when her parents noticed an abnormal gait and increased lumbar hunch. Four months ago she developed a papular rash and swelling of her eyelids followed by a rash on her cheeks. She also developed a red scaly rash over her knees, elbows and knuckles and her parents noticed that the cuticles of her nails were excessively red. Two months ago she began to complain of leg pain and refused to walk up hills. She would no longer brush her hair, and had difficulty dressing and undressing. Over the 2 weeks prior to her admission to hospital she described some difficulty swallowing but there was no choking. She had no history of fevers, but her parents found her more fatigued and her activity level had steadily decreased. In the past 5-12 months she had intermittent "flu-like" symptoms and recurrent abdominal pain. She had no associated vomiting, diarrhea or blood in her stools, however her appetite had decreased.

Survey Monkey

Survey development

Pilot testing

Background

JDM studies

No

Evidence?

Adults

IVIG ✓

MTX/CyA ✓

Fitness Exercise ✓

Leukapheresis/Plasma Exchange ✗

Friends of CARRA



CARRA

Trials

Rare Diseases

Alternative Designs

Plan

1

2

3

Friends of CARRA, TMA, TAS, CIHR, Other

NIH, CIHR, Other?

Survey of current practice

Consensus conference to determine 1 to 3 Rx. protocols

Predictors of Rx response within protocols (incl. pharmacogenomics, etc.)

Comparison of protocols using bias reduction techniques

9 mos

12 mos

24 mos

