# Autism Care Demonstration Outcome Measurement: A Way Forward?

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# Disclosures

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# NASEM Committee Areas Addressed

2. An assessment of the methods used under the demonstration project to measure the effectiveness of applied behavior analysis in the treatment of autism spectrum disorder

Relevance to:

9. The development of a list of recommendations related to the measurement, effectiveness, and increased understanding of the demonstration program and its effect on beneficiaries under the TRICARE program.

# Outline

- ACD Outcome Measures
  - Strengths
  - Weaknesses

- Recommendations for a Better Outcome Assessment Process

- **Major Caveat:** No assessment instrument is perfect. All assessment involves trade-offs. The questions are what tradeoffs should be made AND how do we optimize given the current state of knowledge and available instruments

# Identifying What Matters to Patients / Families

Most currently-available and currently-used clinical measures were not specifically developed for ASD / NDD populations

Many did not use concept elicitation or cognitive interviewing processes or stakeholder engagement

Boateng GO, Neilands TB, Frongillo EA, Melgar-Quinonez HR, Young SL. Best Practices for Developing and Validating Scales for Health, Social, and Behavioral Research: A Primer. *Front Public Health. 2018;6:149.*

Many include items / scales that are not relevant to the full population and/or represent domains or areas of treatment excluded from the TRICARE ACD policy

- Non-verbal / non-speaking people
- Moderate to profound cognitive impairment
- Floor is not low enough

FDA. (2009). *Patient-reported outcome measures: use in medical product development to support labeling claims. (United States Food and Drug Administration, Guidance for Industry.* https://www.fda.gov/downloads/drugs/guidances/ucm193282.pdf

# ASD is associated with a high prevalence of co-occurring medical and mental health conditions

| | Number of datapoints in meta-analysis* | Autism population sample size (n) | Autism population | | General population prevalence (95% CI or SE) | Subgroup moderator analysis | | | | |
|---|---|---|---|---|---|---|---|---|---|---|
| | | | Pooled prevalence (95% CI; 95% PI) | $I^2$ (95% CI; p value†) | | Prevalence in population or registry-based studies (95% CI; 95% PI) | Prevalence in clinical sample-based studies (95% CI; 95% PI) | $R^2$ (QE p value) | $I^2$ (95% CI) | QM p value |
| Attention-deficit hyperactivity disorder | 89 | 210 249 | 28% (25–32; 4–63) | 99·65% (99·55–99·85; <0·0001) | 7·2% (6·7–7·8; point prevalence, aged ≤18 years)[46] | 22% (17–26; 1–55) | 34% (29–39; 7–69) | 2·05% (<0·0001) | 99·64% (99·60–99·84) | 0·0004 |
| Anxiety disorders | 68 | 169 829 | 20% (17–23; 2–48) | 99·53% (99·42–99·87; <0·0001) | 7·3% (4·8–10·9; current prevalence, across ages)[47] | 15% (11–19; 0·5–42) | 26% (22–31; 1–56) | 0% (<0·0001) | 99·54% (99·20–99·85) | 0·0002 |
| Depressive disorders | 65 | 162 671 | 11% (9–13; 0–33) | 99·41% (99·39–99·81; <0·0001) | 4·7% (4·4–5·0; point prevalence of MDD, across ages)[48] | 8% (5–11; 0·01–28) | 14% (11–18; 1–38) | 0·23% (<0·0001) | 99·40% (99·37–99·80) | 0·0003 |
| Bipolar and related disorders | 38 | 153 192 | 5% (3–6; 0–19) | 99·50% (99·40–99·82; <0·0001) | 0·71% (0·56–0·86) for bipolar I; and 0·50% (0·35–0·64) for bipolar II (1-year prevalence, across ages)[49] | 3% (2–5; 0–16) | 7% (4–10; 0–24) | 0·35% (<0·0001) | 99·50% (99·48–99·81) | 0·018 |
| Schizophrenia spectrum and psychotic disorders | 42 | 166 627 | 4% (3–5; 0–14) | 99·18% (99·00–99·87; <0·0001) | 0·46% (0·41–0·50; 1-year prevalence, across ages)[50] | 2% (1–4; 0–11) | 7% (4–9; 0–19) | 0% (<0·0001) | 99·18% (99·01–99·84) | 0·0004 |
| Obsessive-compulsive and related disorders | 47 | 53 243 | 9% (7–10; 1–21) | 96·85% (96·75–99·87; <0·0001) | 0·7% (0·4–1·1; 1-year prevalence, aged ≥18 years)[51] | 4% (2–6; 0–13) | 12% (10–15; 3–26) | 12·51% (<0·0001) | 96·20% (96·17–99·37) | <0·0001 |
| Disruptive, impulse-control, and conduct disorders | 50 | 140 946 | 12% (10–15; 0–36) | 99·52% (99·47–99·90; <0·0001) | 8·9% (SE 0·5; 1-year prevalence, aged ≥18 years)[52] | 7% (4–10; 0–28) | 22% (17–27; 3–50) | 0% (<0·0001) | 99·53% (99·42–99·88) | <0·0001 |
| Sleep–wake disorders | 26 | 190 963 | 13% (9–17; 0–43) | 99·87% (99·78–99·93; <0·0001) | 3·7% (NA; 1-year prevalence, aged ≤18 years)[53] | 11% (7–17; 0–39) | 16% (8–25; 0–47) | 8·52% (<0·0001) | 99·85% (99·77–99·91) | 0·356 |

Lai MC, Kassee C, Besney R, et al. Prevalence of co-occurring mental health diagnoses in the autism population: a systematic review and meta-analysis. Lancet Psychiatry. Oct 2019;6(10):819-829. doi:10.1016/S2215-0366(19)30289-5

# Domains / Constructs to Consider for ABA Outcome Assessment

| Autism Symptoms | Functioning | Other Concerns | Well-Being |
|---|---|---|---|
| Social Communication / Interaction | Daily Living Skills / Practical Functioning | Anxiety | Satisfaction with Therapy |
| Restricted/Repetitive Behavior | Motor Skills | Attention / ADHD | Quality of Life / Flourishing |
| ASD Subdomains | Leisure Skills | Irritability / Challenging Behavior | External Environmental Supports |
| | Executive Functioning | Mood / Affective Experience | Access to Medical and Behavioral Health Services |
| | Language / Speech | Sleep Problems | |
| | Family Functioning | GI Problems | |
| | | Feeding Challenges | |

Red = very weak or no coverage

Orange = some coverage but not strong measurement

# Modern Understanding of Core ASD Symptoms

Social Communication / Interaction

- Basic social communication (verbal and non-verbal)
- Social motivation / affiliation
- Perspective taking
- Relationships

- Restricted / Repetitive Behavior
  - Repetitive motor and speech
  - Insistence on sameness
  - Restricted interests
  - Sensory interests
  - Sensory sensitivities


Phillips JM, et. al. *Mol Autism. 2019;10:48.*

Uljarevic M. et. al. *J Am Acad Child Adolesc Psychiatry. 2020;59(11):1252-1263 e1253.*

Uljarevic M. et. al. *J Clin Child Adolesc Psychol. 2021;50(5):609-618.*

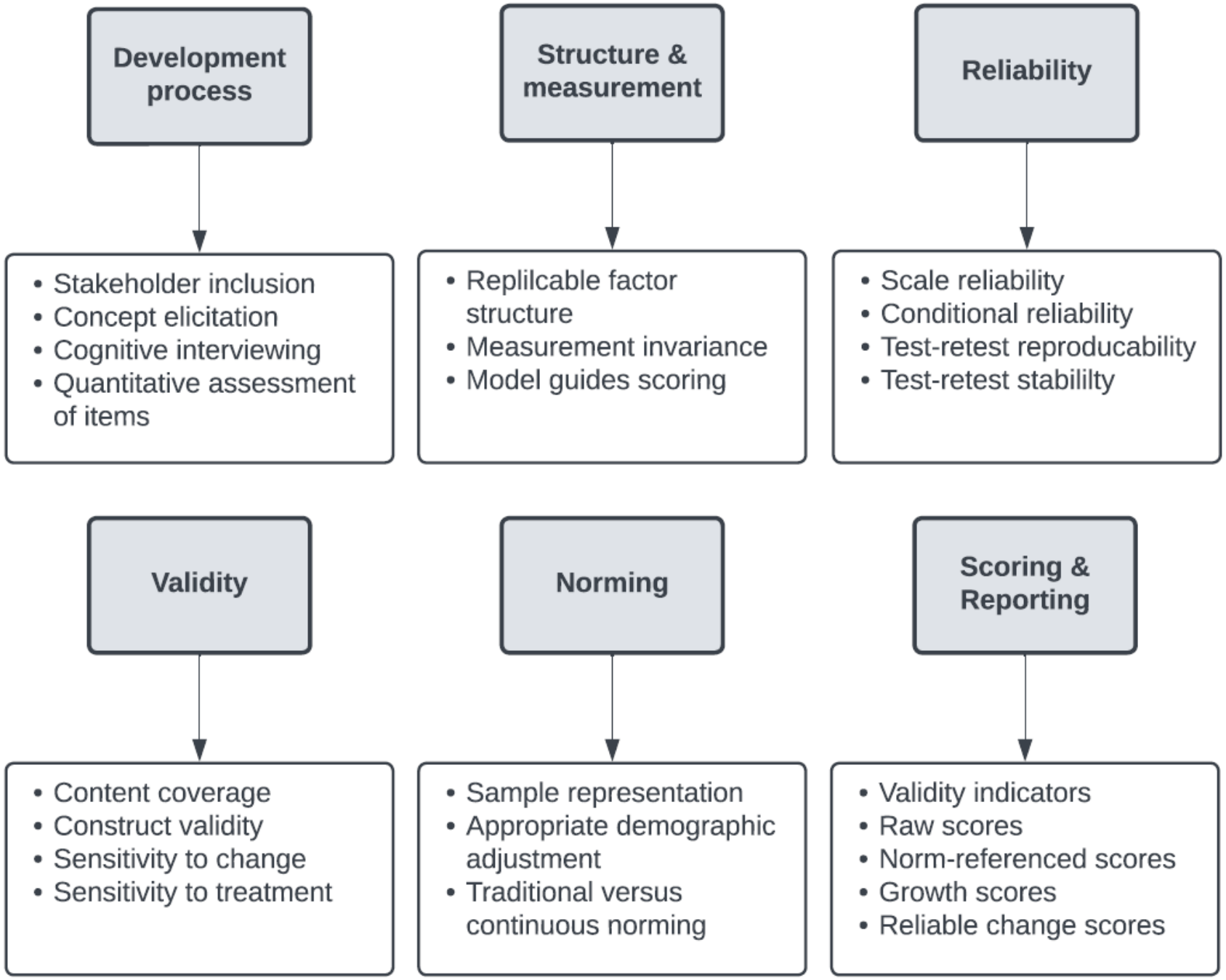
Frazier TW, et. al. *Autism. 2017;21(6):749-759.*

Uljarevic M, et. al. *J Am Acad Child Adolesc Psychiatry. 2023;62(5):568-581.*

Uljarevic M. et. al. *J Am Acad Child Adolesc Psychiatry. 2022;61(3):446-457.*

Frazier TW, *Autism. 2014;18(1):31-44.*

# Proposed Psychometric Criteria for Outcome Measure Prioritization



# Proposed Practical Criteria

i. Automated administration

ii. Automated scoring

iii. Visual display of measure results

iv. Automated interpretative statements, with clinician selection/modification

v. Automated connection of results with intervention target identification, with clinician selection/addition/modification

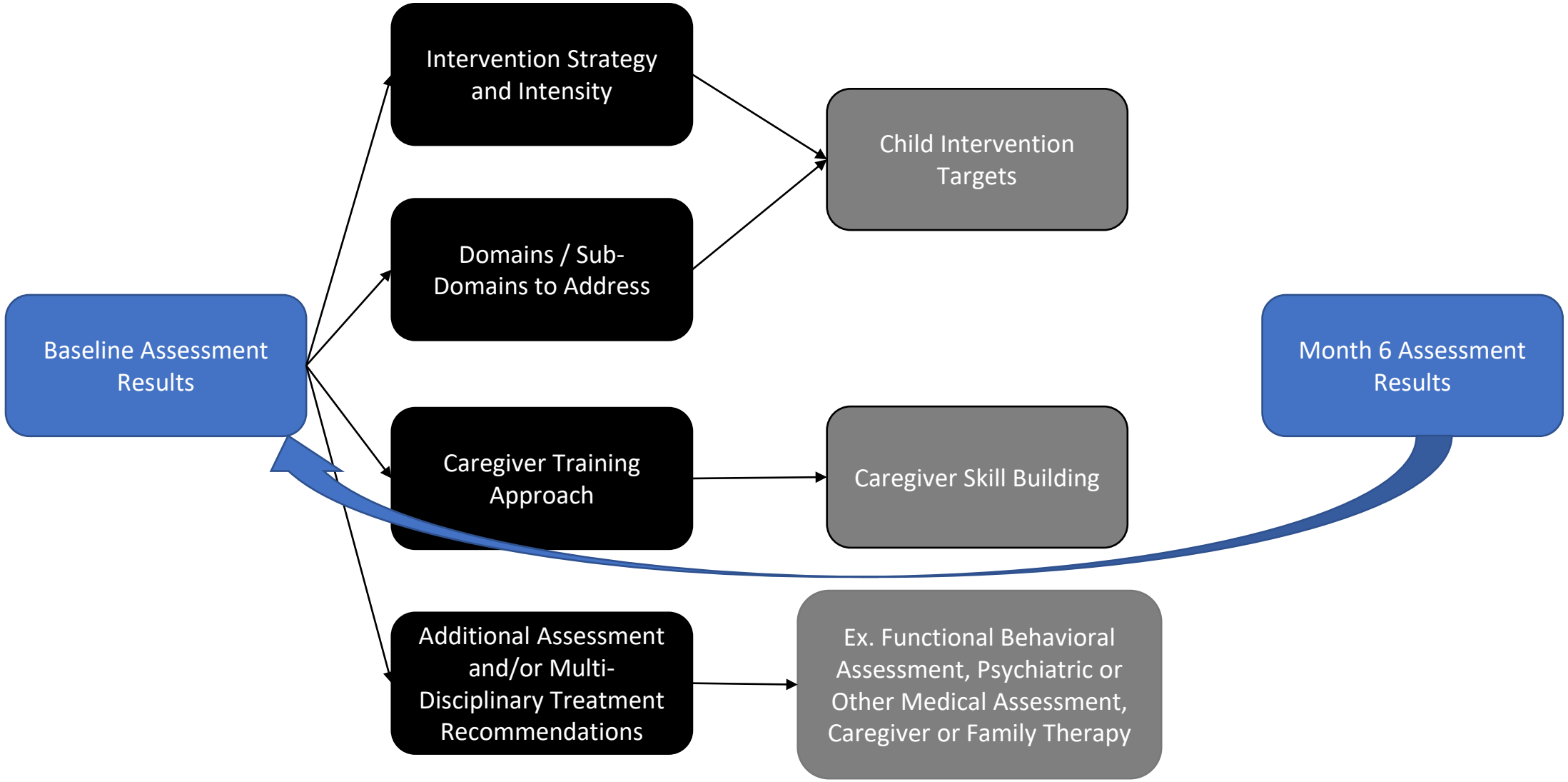
vi. Automated clinical guidance for additional assessment, intervention strategy, and referral with clinician selection/modification

vii. Automated progress monitoring

viii. Connection of target identification with goals/objectives and teaching plan selection

ix. Aggregation of measures and reporting to facilitate clinical workflow

# Assessment-Driven Treatment



# Current DoD / TRICARE Outcome Assessment Battery

Note: In interest of time, I cannot review every detail of the instruments and am only covering the major strengths and weaknesses applicable in this context

# Vineland-3

## Strengths

- Long history of clinical and research use
- Massive amount of evidence of construct validity
- Wide coverage of developmental and functional levels
- Growth scores useful for tracking change
- Content coverage
  - Daily living skills
  - Social interaction
  - Communication
  - Motor skills
    - Some coverage of internalizing and externalizing psychopathology

## Weaknesses

- Long administration time (40+ minutes)
- Not built for ABA outcome assessment, stakeholders not involved in measure construction
- Social and communication sections do not measure core ASD symptom domains
- Small number of studies of the factor structure suggests instrument may not scored correctly
- Behavior coverage is very good but not sufficient for ABA intervention planning
- Not all sections are necessary for all cases
- Not all items are applicable to all ASD cases
- Test publisher maintains tight control of electronic administration, without potential to automate administration and databasing of data with other measures

# SRS-2

## Strengths

- Long history of clinical and research use
- Parent- and teacher-report
- Includes SCI and RRB symptoms
- Measures across a wide range of autistic traits
- Total score has excellent psychometric properties
- Has shown sensitivity to change
  - Although may be susceptible to rater and placebo effects

## Weaknesses

- Not built for ABA outcome assessment, stakeholders not involved in measure construction
- Does not measure all ASD symptom sub-domains
  - RRB coverage is very weak
- Limited utility for intervention planning
- Not all items are applicable to all ASD cases
  - require speech or imply higher levels of cognitive function
- Test publisher maintains tight control of administration, with limited potential to automate administration and databasing of data with other measures

# PDDBI

## Strengths

- Parent and teacher forms
- Includes a wide range of core autism and associated symptoms
- Covers anxiety and aggression
- Covers receptive and expressive aspects of communication
- Substantial prior use in ASD intervention studies
- Content has utility for intervention planning

## Weaknesses

- Published in 2003
  - Most published papers are not examining psychometric properties
- 30+ minute administration
- Not built for ABA outcome assessment, stakeholders not involved in measure construction
- Factor structure is not well understood
  - Unclear scoring
- Mixed evidence of sensitivity to change in behavioral intervention studies
- Which scores to use for tracking change?
- Test publisher maintains tight control of administration, with limited potential to automate administration and databasing of data with other measures

# PSI-4 / SIPA

## Strengths

- Long history of clinical and research use
- Massive literature
  - Google Scholar – 1470 results
- Total score has good reliability and construct validity evidence
- Strong coverage of content areas related to parent feelings of competence and stress, including child characteristics
- Prior evaluations in similar populations

## Weaknesses

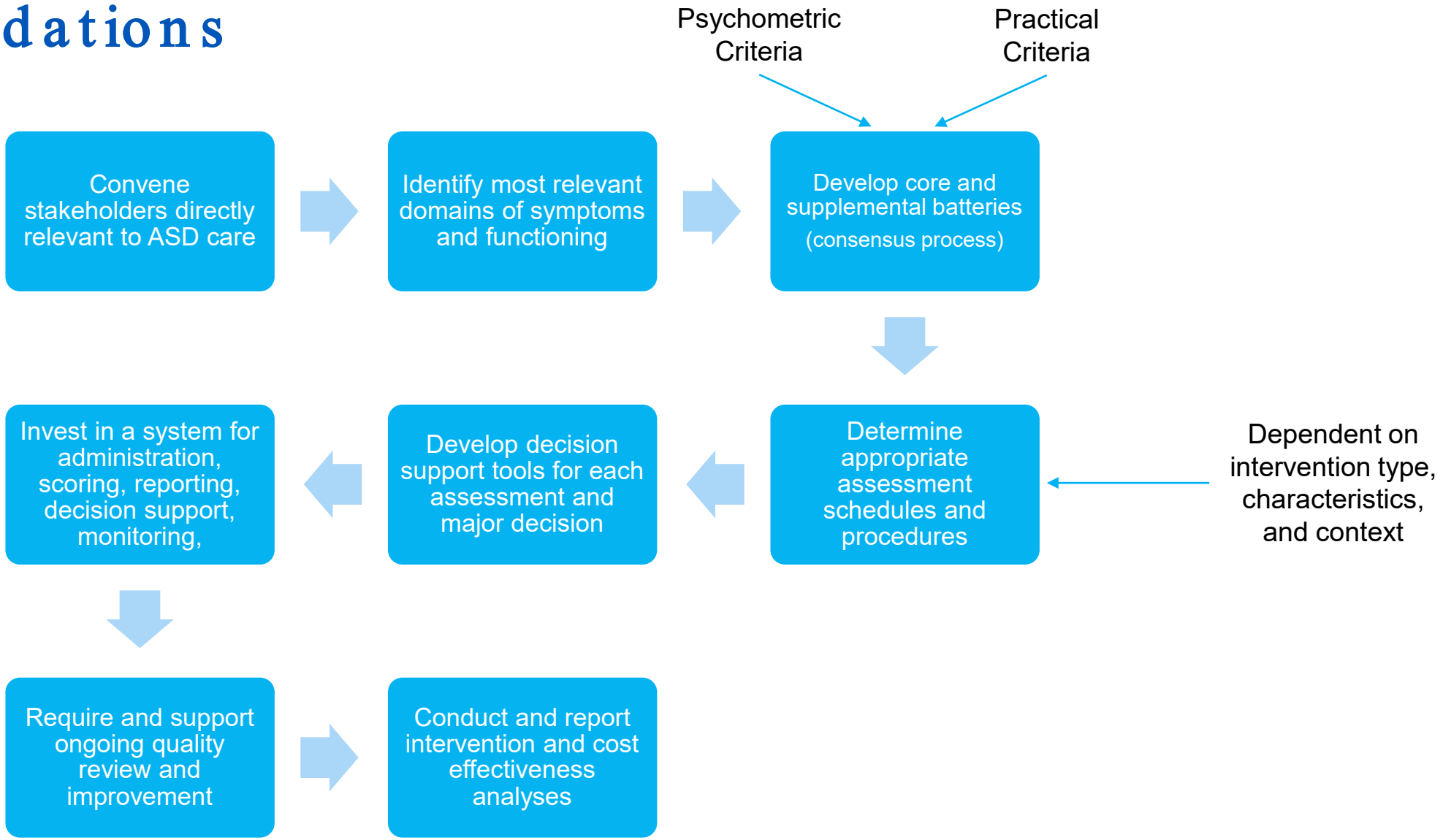
- 120 / 112 items
- 20-30 minute administration
- Significant differences in content across forms
- Not built for ABA outcome assessment, stakeholders not involved in measure construction
- Factor structure is not well replicated
  - Unclear scoring
- No evidence of sensitivity to change in behavioral intervention
- Test publisher maintains tight control of administration, with limited potential to automate administration and databasing of data with other measures

# Summary

ACD Battery has significant limitations that necessitate a revision in thinking about the optimal outcomes assessment approach

- Not built for context, no stakeholder input, and with mixed psychometric properties
- Does not adequately assess all of core autism sub-domains that have been identified
  - Social motivation, sensory sensitivity, sensory interests
- Construct overlap
  - Example. Basic social communication over-sampled
- Limited coverage of co-occurring conditions relevant to child functioning
- Coverage of parent stress-related construct but lacking broader coverage of child/family/ecosystem quality of life
- Very little ability to deliver in an automated fashion as a battery and collect and store information for clinical quality improvement and ongoing evaluation

# Recommendations

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