Conceptual model
of core digital measures for pediatric rare diseases

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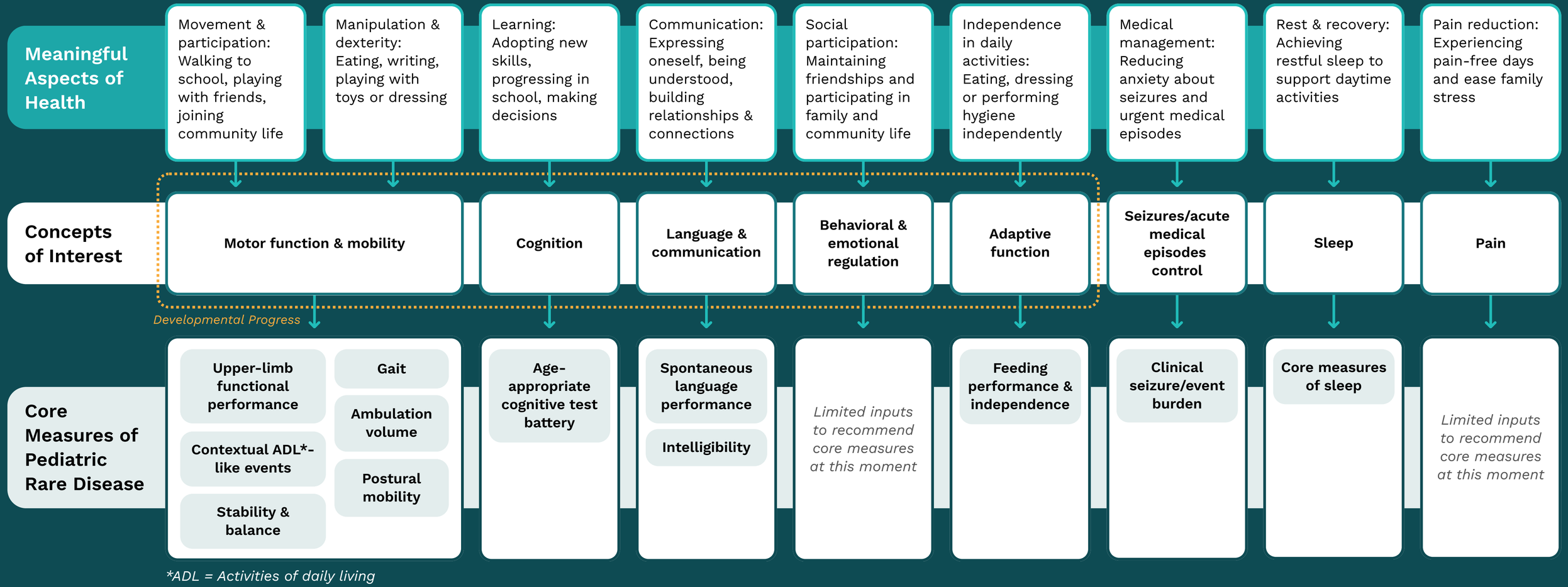

*ADL = Activities of daily living

Digital clinical measures | Activity, mobility & motor function

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Ambulation volume

DESCRIPTION
Tracks the total amount of walking a child does in a day, including their step count and how much time they spend moving around versus resting.

ENDPOINTS TO CHOOSE FROM
Step count, # of walking bouts, walking duration.

Contextual ADL-like events

DESCRIPTION
Measures how well a child performs daily tasks (ADL = activities of daily living), such as feeding.

ENDPOINTS TO CHOOSE FROM
Activities such as occupational use, object/toy manipulation, feeding-related hand use, self-care hand use; measuring activity frequency, duration, success rate, laterality/symmetry, workspace volume.

Feeding performance & independence

DESCRIPTION
Assesses how well a child can eat and drink on their own, looking at their ability to swallow, use utensils, and feed themselves without spilling.

ENDPOINTS TO CHOOSE FROM
Swallowing ability, texture level achieved, self-feeding attempts/min, % bites self-initiated, spill rate, utensil use success %, # independent sips/bites.

Gait

DESCRIPTION
Analyzes the quality and pattern of a child's walk such as stride, speed, and how smooth or symmetrical their movement is.

ENDPOINTS TO CHOOSE FROM
Stride length/velocity/time variability, cadence, gait symmetry, swing time, stance time, double-support time, trunk sway, toe-off angle, stride-to-stride variability, composite scores (e.g. SV95C = stride velocity 95th centile), walking speed, vertical ground reaction force, movement smoothness, stride temporal autocorrelation.

Postural mobility

DESCRIPTION
Evaluates how easily and frequently a child can change body positions, such as standing up from a chair, sitting down, or climbing stairs.

ENDPOINTS TO CHOOSE FROM
Frequency / exposure of postural transitions (e.g., sit to stand transition/day, stair ascents/day), transitions duration, transition mechanics, postural sway.

Stability & balance

DESCRIPTION
Measures how steady a child is when sitting, standing or walking, how well they can hold their head up, control their trunk, or stay upright without falling.

ENDPOINTS TO CHOOSE FROM
Non-ambulatory children: Head hold, trunk sway, time unsupported, loss of balance, supported standing. Ambulatory children: step width variability, gait symmetry, fall/near-fall/loss-of-balance rate, root-mean-square acceleration, jerk.

Upper limb functional performance

DESCRIPTION
Analyzes the general movement ability of the arms and hands, such as reaching distance, speed, and range of motion, regardless of the specific task being performed.

ENDPOINTS TO CHOOSE FROM
Use volume (time active/day), acceleration, angular velocity, upper-limb workspace volume, hand-use ratio, range of motion, jerk.

Digital clinical measures | Sleep, seizures & acute medical events

Clinical seizure / acute event burden

DESCRIPTION
Records the overall impact of seizures or medical episodes by tracking how often they happen, whether they occur at night, and their severity and details using (digital) diaries and passive sensor signals.

ENDPOINTS TO CHOOSE FROM
Event frequency: count per 28 days, counts year, % occurring during sleep vs daytime. Events can be recorded via 1) a self-report (diary), to include: timestamp, type, duration, triggers, clustering, rescue medication use, hospitalization; or 2) sensor-based digital health technologies that infer event frequency, duration, severity and recovery time. Digital measures include electroencephalogram, accelerometer, photoplethysmography, electrodermal activity, peripheral oxygen saturation, heart rate variability, body temperature, electromyography, and audio & video.

Core measures of sleep

DESCRIPTION
Uses sensors-based digital health technologies to objectively track sleep quality, including how long a child sleeps, when they wake up, and how often their sleep is interrupted.

ENDPOINTS TO CHOOSE FROM
Total sleep time (TST, duration), wake after sleep onset (WASO, duration), initial sleep onset latency (SOL, duration), number of wake events in the primary sleep period (count), sleep efficiency (%), total napping time (duration). Dive deeper into therapeutic area-agnostic core measures of sleep.

Digital clinical measures | Thinking, behavior & communication

Age-appropriate cognitive test battery

DESCRIPTION
Uses game-like tasks on a tablet or phone to test thinking skills suited for the child's age, such as memory, attention span, and processing speed.

ENDPOINTS TO CHOOSE FROM
For children >2 years: quantifying change across core cognition domains with age/ability-appropriate game-like tasks delivered on tablet/phone/PC. Cognition domains: attention, working memory, processing speed, executive function, learning & memory. Endpoints include: accuracy (%), reaction time (ms), success rates, etc.

Intelligibility

DESCRIPTION
Evaluates how clearly a child speaks, capturing how easily they are understood when speech is affected by muscle weakness, motor coordination issues, or developmental delays.

ENDPOINTS TO CHOOSE FROM
ASR (automatic speech recognition)-assisted percent intelligible words (PIW) or % intelligible utterances.

Spontaneous language performance

DESCRIPTION
Captures a child's natural speech in their everyday environment, tracking things like how often they vocalize, how fast they speak, or the variety of words they use.

ENDPOINTS TO CHOOSE FROM
Language productivity: frequency of child vocalizations, mean length of utterance (MLU), total # of utterances, speech rate; lexical diversity: number of different words (NDW), moving-average type-token ratio (MATTR), measure of textual lexical diversity (MTLD), hypergeometric distribution D (HD-D); morphosyntactic accuracy & complexity.

Impacts on caregivers, families and siblings

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Meaningful Aspects of Health

Concepts of Interest

Core Measures of Pediatric Rare Disease

Changes in MAH and COI: Impacts on caregivers, families and siblings

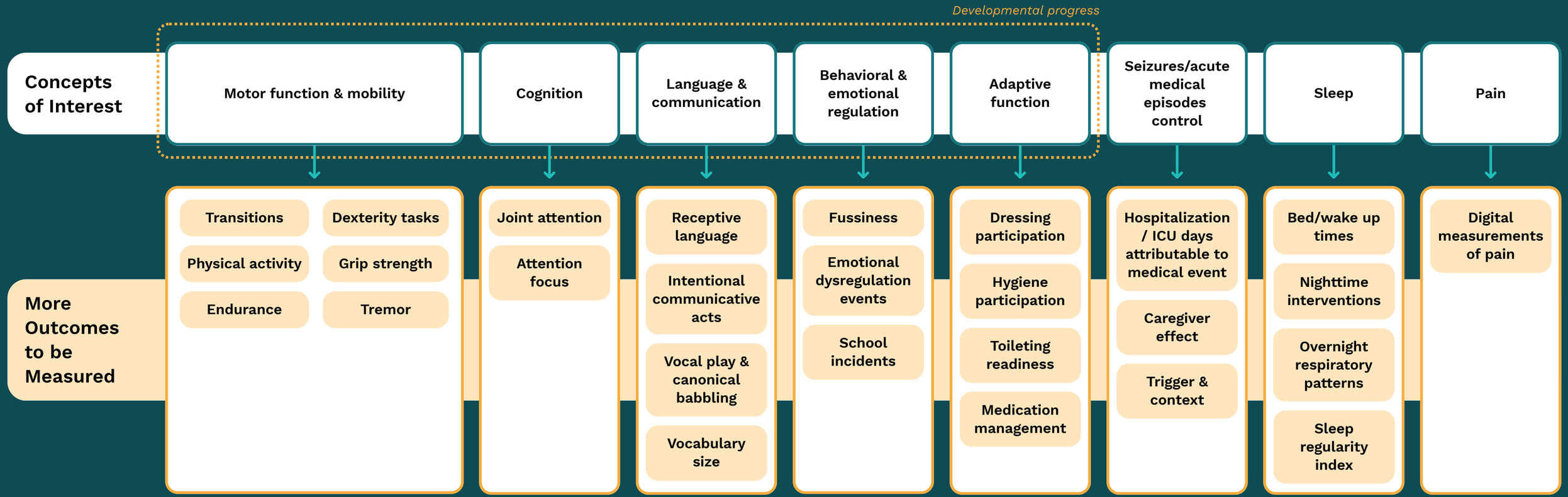
Hypervigilance	Financial burden, loss of income	Social isolation & relationships strain
Fragmented sleep	Home & school accommodations	Anxiety & fear of seizures & medical events
Care coordination	Safety management	Concern about long-term caregiving
Stress & fatigue	Constant ADL dependence	Sibling relationship & role strain
Polypharmacy management	Negative impact on mental health, trauma	Inability to communicate with the child

Example measurable treatment effects impacting caregivers, families, and siblings

Improvement in motor function & mobility	Positive progress in attention, learning & problem-solving	Reduced overall seizure/acute medical event burden
Increased independence in daily tasks	Increased time spent active	Consolidated, restorative sleep
Improved stability & balance	Improved daily routines	Progression of communication abilities

More outcomes to be measured

This section includes outcomes that did not achieve the level of consensus required for the core measures set. While supported by evidence and expert opinion, these outcome measures are either too condition-specific or may require further research and technological maturation.



Conceptual model of core digital measures for pediatric rare diseases

To learn more about this conceptual model, the measures, and the dataset, visit:

- Core digital measures of pediatric rare diseases

Additional quicklinks

- Project home
- Implementation guide
- Customize your measures

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