Supplemental Materials Part 1

Reproducibility in small-N treatment research in aphasia and related disorders: a tutorial

Introduction

This document details the code needed to reproduce the analysis in section 1 of the manuscript. For batch calculations of effect sizes across participants see part 2.

Setup

Load packages and functions

```
# Uncomment and run this line to install packages if needed
# install.packages(c("here", "SingleCaseES", "lme4",
# "emmeans", "brms", "tidybayes", "qqdist", "tidyverse"))
# Instructions for installing RStan are here:
# https://qithub.com/stan-dev/rstan/wiki/RStan-Getting-Started
library(here)
                         # for locating files
library(SingleCaseES) # calculating SMD, Tau-U
library(lme4)
library(emmeans)
                         # frequentist mixed-effects models
                         # estimating effect sizes from lme4
library(brms)
                         # bayesian mixed-effects models
library(brms) # bayesian mixed-effects models
library(tidybayes) # estimating effect sizes from brms
                         # Visualizing posterior distributions
library(ggdist)
library(tidyverse)
# set a seed for reproducibility
set.seed(42)
```

Read in data

Note that the current setup uses RStudio R projects (https://support.rstudio.com/hc/en-us/articles/200526207-Using-RStudio-Projects). One of the features of R projects is that the working directory is automatically set to the project root (the folder with the .Rproj). A discussion of R projects can be found at https://www.tidyverse.org/blog/2017/12/workflow-vs-script/. In this case here("study-data") refers to the /study-data folder inside the project.

```
# map_df takes a function, in this case read_csv().
# show_col_types suppresses output since we're reading in many files
df <- files %>%
  map_dfr(read_csv, show_col_types = FALSE)
```

Preview the data

```
head(df)
## # A tibble: 6 x 11
##
     participant condition phoneme itemType phase
                                                        session item trials spt2017
                  <chr>
                                                           <dbl> <chr>
                                                                         <dbl> <chr>
     <chr>>
                            <chr>>
                                     <chr>
                                               <chr>
## 1 P1
                  blocked
                            pr
                                     tx
                                               baseline
                                                               1 pr-1
                                                                            10 pre
## 2 P1
                  blocked
                                               baseline
                                                               1 pr-12
                                                                            10 pre
                                     tx
                            pr
## 3 P1
                                               baseline
                  blocked
                                     tx
                                                               1 pr-4
                                                                            10 pre
                            pr
## 4 P1
                  blocked
                                               baseline
                                                               1 pr-15
                                                                            10 pre
                                     tx
                            pr
## 5 P1
                                               baseline
                  blocked
                                                               1 pr-5
                                                                            10 pre
                            pr
                                     tx
## 6 P1
                  blocked
                                               baseline
                            pr
                                     tx
                                                               1 pr-7
                                                                            10 pre
## # ... with 2 more variables: response <dbl>, n_baselines <dbl>
```

Table 1: Data variables and their description

Variable	Description
participant condition phoneme itemType phase	de-identified participant ID probe schedule (blocked or random) target_phoneme item condition (treatment or generalization) treatment phase
session item trials spt2017 response	session number from Wambaugh 2017 item identifier number of items in the list (per phoneme) phase used to calcualte effect sizes in Wambaugh et al., 2017 accuracy of participant response
$n_baselines$	Number of baseline sessions

Case example: Participant 10

Filter data for Participant 10

Starting from the entire dataset, filter for participant 10, treated items, and the blocked condition. Then to calculate session-level data (the number of correct responses per session), group by session, and use the summarize function to calculate the number of correct responses per session. The group_by function also includes phase and spt2017 because we want to keep these variables in the summary data frame, but their addition doesn't affect grouping. The .groups argument removes the grouping after summarize.

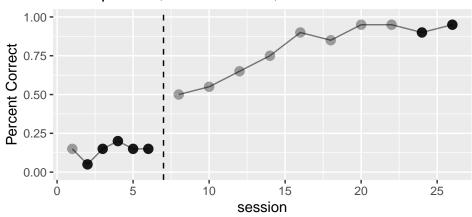
```
# and spt2017
group_by(session, phase, spt2017) %>%
# calculates the number of correct responses for each level in group_by
# the .groups argument removes the grouping after summarize
summarize(sum_correct = sum(response), .groups = "drop")
```

Plot performance over time

Plotting data from participant 10. First, we select only the baseline and treatment phases (ignoring the washout and maintenance phases for the purpose of this paper). The we create a dummy variable reflecting whether or not the session was included in the SMD/PMG calculations. Finally, we use the {ggplot2} package to plot the data. A recent primer on {ggplot2} for researchers unfamiliar with R can be found here: https://doi.org/10.1177/25152459221074654

```
p1 = P10 \%
  # filter for baseline and treatment phases
  filter(phase == "baseline" | phase == "treatment") %>%
  # create a new variable called Measure that has a value of
  # include if spt2017 is not an NA value and exclude if it is.
  # the levels argument indicates that exclude should be
  # the first level of the factor.
  mutate(Measure = factor(
   ifelse(!is.na(spt2017), "include", "exclude"),
           levels = c("exclude", "include"))) %>%
  # create a plot with session on the x axis, percent
  # correct on the y axis, and group by phase
  ggplot(aes(x = session, y = sum_correct/20, group = phase)) +
  # add points to the graph
  geom_point(aes(alpha = Measure), size = 3) +
  # add a line to the graph
  geom_line(alpha = 0.5) +
  # add a vertical line where x = 7
  geom_vline(aes(xintercept = 7), linetype = "dashed") +
  scale_x_continuous(breaks = seq(0,30,5)) +
  ylim(0, 1) +
  scale_alpha_discrete(range = c(0.35, 0.9)) +
  labs(title = "Participant 10, treated words, blocked condition",
       caption = "Dark circles represent data points used to calculate
       the within-case standardized mean difference in Wambaugh et al.,
       (2017)",
       y="Percent Correct") +
  guides(alpha = "none")
p1
```

Participant 10, treated words, blocked condition



Dark circles represent data points used to calculate the within–case standardized mean difference in Wambaugh et al., (2017)

Within-case standardized mean difference

There are any number of ways to calculate the within case standardized mean difference using R code. In this example, we have used the SMD() function from the established package {SingleCaseES} by James Pustejovsky because it includes additional functions that may be of interest to researchers in aphasiology.

Additionally, we do not show all information returned by the function, which also includes a 95% confidence interval, as it is not clear that this confidence interval applies to the $d_{\rm BR}$ modification of the original within-case standardized mean difference.

[1] 11.46566

Proportion of potential maximal gain

There is no R package that includes a function to calculate PMG to our knowledge. However, creating such a function is relatively straightforward. A function that calculates PMG similar to the SMD() function from the $\{\text{SingleCaseES}\}\$ package might take the following form, with an additional argument for the number of items treated (nitems). The function calculates the mean of the A phase and B phase, and then calculates and returns the PMG value from the same data as d_{BR} above.

```
# the function is named PMG and takes 3 arguments:
# vectors of the a_data and b_data, and
# a single number indicating how many items were treated
PMG <- function(a_data, b_data, nitems){
   mean_a <- mean(a_data) # calculate mean of a_data
   mean_b <- mean(b_data) # calculate mean of b_data
   pmg <- (mean_b-mean_a)/(nitems-mean_a) # calculate PMG
   return(pmg) # return the PMG value.
}

PMG(a_data = A, b_data = B, nitems = 20)</pre>
```

Tau-U

The Tau-U family of effect sizes (and several other non-overlap measures) can be calculated using the $\{\text{SingleCaseES}\}\$ package. In this case, we use all data summarized in the P10 data frame (and not just the data used to calculate d_{BR}).

First, we estimate the trend line during the baseline phase, which can be generated by creating a simple linear model using the lm() function. The model includes the number of correct responses as the dependent variable and the session number as the independent variable. The session coefficient reflects the slope during the baseline phase. The coef() function simple extracts the model coefficients.

```
P10 %>%
  filter(phase == "baseline") %>%
  lm(data = ., sum_correct~session) %>%
  coef()
```

```
## (Intercept) session
## 2.133333 0.200000
```

The Tau() and Tau_U() functions take the same data structure as the SMD() and PMG() functions above.

Using the conservative benchmark of 0.33 recommended by Lee and Cherney (2018), we would calculate $Tau_{Avs.B}$ as the slope of the baseline phase is only 0.2 (from the result above). To calculate $Tau_{Avs.B}$, we can use the Tau() function.

```
A = P10 %>% filter(phase == "baseline") %>% pull(sum_correct)
B = P10 %>% filter(phase == "treatment") %>% pull(sum_correct)
Tau(A_data = A, B_data = B)
```

```
## ES Est SE CI_lower CI_upper ## 1 Tau 1 0.02710291 1 1
```

However, if we had elected to correct for baseline trends and use $Tau_{Avs.B-trendA}$, we can use the similar $Tau_{U}()$ function.

```
Tau_U(A_data = A, B_data = B)
## ES Est
## 1 Tau-U 0.95
```

Mixed-effects model-based effect sizes

The mixed-effects model example for participant 10 uses item-level data, so we need to create a new dataframe for this model. As discussed in the manuscript, the model formula is based on a structure from Huitema & McKean (2000).

$$Y_t = \beta_0 + \beta_1 T_t + \beta_2 D_t + \beta_3 [T_t - (n_1 + 1)] D_t + \epsilon_t$$

The DV is predicted by the model intercept, baseline slope, level change, and slope change parameters.

After selecting data from participant 10, the coefficients are created by:

- setting baseline_slope equal to the session variable
- level_change is a dummy variable, 0 during baseline and 1 during treatment

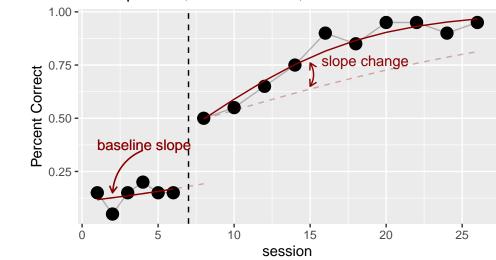
• slope_change is created by subtracting the number of baselines plus 2 from the baseline slope value, and then multiplying the result with the level_change variable. Typically, if probing every session, the formula calls for subtracting the number of baselines plus 1. However, because Wambaugh et al., (2017) used intermittent probing schedules, and probed every other treatment session starting at the second, we need to add 2 to the number of baselines to ensure that the slope change variable starts at 0 on the first recorded treatment probe.

The resulting matrix looks as follows:

phase	baseline_slope	level_change	slope_change
baseline	1	0	0
baseline	2	0	0
baseline	3	0	0
baseline	4	0	0
baseline	5	0	0
baseline	6	0	0
treatment	8	1	0
treatment	10	1	2
treatment	12	1	4
treatment	14	1	6
treatment	16	1	8
treatment	18	1	10
treatment	20	1	12
treatment	22	1	14
treatment	24	1	16
treatment	26	1	18

Manuscript Figure 2. visualizes each parameter in this model structure. The code can be found in the .Rmd file, and is omitted from the pdf due to its length.

Participant 10, treated words, blocked condition



The model formula can be expressed as follows:

$$\begin{aligned} \operatorname{response}_{i} &\sim \operatorname{Binomial}(n=1, \operatorname{prob}_{\operatorname{response}=1} = \widehat{P}) \\ \log \left[\frac{\widehat{P}}{1 - \widehat{P}} \right] &= \alpha_{j[i]} + \beta_{1j[i]} T_{t} + \beta_{2j[i]} D_{t} + \beta_{3j[i]} [T_{t} - (n_{1} + 1)] D_{t} \\ \left(\begin{matrix} \alpha_{j} \\ \beta_{1j} \\ \beta_{2j} \\ \beta_{3j} \end{matrix} \right) &\sim N \left(\begin{matrix} \mu_{\alpha_{j}} \\ \mu_{\beta_{1j}} \\ \mu_{\beta_{2j}} \\ \mu_{\beta_{3j}} \end{matrix} \right), \begin{pmatrix} \sigma_{\alpha_{j}}^{2} & \rho_{\alpha_{j}\beta_{1j}} & \rho_{\alpha_{j}\beta_{2j}} & \rho_{\alpha_{j}\beta_{3j}} \\ \rho_{\beta_{1j}\alpha_{j}} & \sigma_{\beta_{1j}}^{2} & \rho_{\beta_{1j}\beta_{2j}} & \rho_{\beta_{1j}\beta_{3j}} \\ \rho_{\beta_{2j}\alpha_{j}} & \rho_{\beta_{2j}\beta_{1j}} & \sigma_{\beta_{2j}}^{2} & \rho_{\beta_{2j}\beta_{3j}} \\ \rho_{\beta_{3j}\alpha_{j}} & \rho_{\beta_{3j}\beta_{1j}} & \rho_{\beta_{3j}\beta_{2j}} & \sigma_{\beta_{3j}}^{2} \end{pmatrix}, \text{ for item } \mathbf{j} = 1, \dots, \mathbf{J} \end{aligned}$$

The following shows how we arrived at the final model for P10

1. First, we fit the maximal random effects structure. However, the model did not converge.

2. Second, we tried specifying a different optimizer, following recommendations that can be found at https://bbolker.github.io/mixedmodels-misc/glmmFAQ.html#convergence-warnings.

We can now examine the model summary:

```
## Generalized linear mixed model fit by maximum likelihood (Laplace)
```

```
## Generalized linear mixed model fit by maximum likelihood (Laplace
## Approximation) [glmerMod]
## Family: binomial ( logit )
```

```
## Formula: response ~ baseline_slope + level_change + slope_change + (1 +
##
       baseline_slope + level_change + slope_change | item)
##
   Control: glmerControl(optimizer = "bobyqa")
##
##
##
                 BIC
                        logLik deviance df.resid
        AIC
      249.5
##
               302.3
                        -110.8
                                   221.5
                                              306
##
##
   Scaled residuals:
##
       Min
                 1Q
                    Median
                                 3Q
                                         Max
##
   -2.2995 -0.2462
                     0.0202
                             0.1563
                                      3.6534
##
##
   Random effects:
    Groups Name
##
                           Variance Std.Dev. Corr
##
    item
            (Intercept)
                           4.5354
                                     2.1296
##
           baseline_slope 0.3358
                                     0.5795
                                              -0.75
##
                                     2.3773
                                               0.34 - 0.01
           level_change1
                           5.6514
##
                           0.8646
                                     0.9298
                                               0.66 -0.97 -0.14
           slope_change
  Number of obs: 320, groups: item, 20
##
##
##
  Fixed effects:
##
                   Estimate Std. Error z value Pr(>|z|)
## (Intercept)
                   -2.70900
                               1.33242
                                         -2.033
                                                    0.042 *
## baseline slope
                   0.01743
                               0.33249
                                          0.052
                                                    0.958
## level change1
                    2.50785
                               1.69431
                                          1.480
                                                    0.139
## slope_change
                    0.39216
                               0.39651
                                          0.989
                                                    0.323
##
                   0 '***' 0.001 '**' 0.01 '*' 0.05 '.' 0.1 ' ' 1
## Signif. codes:
##
## Correlation of Fixed Effects:
##
                (Intr) bsln_s lvl_c1
## baselin_slp -0.861
  level_chng1
               0.535 - 0.723
## slope_chang 0.761 -0.916
                               0.499
```

We note that in this case, further reducing the random effects structure often returns a significant result for the level_change parameter, demonstrating how our choice of random effect structure can influence the statistical significance of model parameters. For this reason, we stress that researchers are forthcoming of the model modifications made from the initial maximal random effect structure and also report what modifications result in parameters changing in significance.

Calculating an overall effect size for this participant requires contrasting performance either at the end of treatment with and without the level change and slope change parameters, or contrasting performance at the end of treatment with performance at the end of baseline. The former option assumes that any baseline trend would have continued throughout the treatment phase in the absence of treatment, is typically more conservative.

While there is a small, empirical baseline slope in this data, it may be reasonable to consider that this slope is largely driven by lower performance on the second probe session, and that performance in baseline sessions 3-6 are stable, and therefore estimate the difference in performance from the end of baseline to the end of treatment. Criteria for such decisions should ideally be made a-priori if possible, and reported in publications regardless.

1. First, we generate the marginal means for each combination of baseline slope, level change, and slope change.

```
##
    baseline_slope level_change slope_change emmean
                                                        SE df asymp.LCL asymp.UCL
##
                 7 0
                                            0 -2.5870 1.36 Inf
                                                                   -5.252
                                                                             0.0782
                26 0
                                                                  -17.009
                                                                            12.4976
##
                                            0 -2.2559 7.53 Inf
##
                 7 1
                                            0 -0.0792 1.20 Inf
                                                                   -2.427
                                                                             2.2684
                                                                  -12.263
##
                26 1
                                            0 0.2520 6.39 Inf
                                                                            12.7672
##
                 7 0
                                           19 4.8641 6.46 Inf
                                                                   -7.801
                                                                            17.5290
##
                26 0
                                           19 5.1952 3.06 Inf
                                                                   -0.802
                                                                            11.1928
                 7 1
                                                                   -7.041
##
                                           19 7.3719 7.35 Inf
                                                                            21.7846
##
                26 1
                                           19 7.7030 2.49 Inf
                                                                    2.819
                                                                            12.5873
##
```

- ## Results are given on the logit (not the response) scale.
- ## Confidence level used: 0.95
 - 2. This returns a table of all possible comparisons, and we are only interested in contrasting the first row (beginning of treatment) with the last row (end of treatment). After selecting these two rows, we can then contrast their estimates.

```
# code to select first and last rows
# The 1 indicates that the row should be selected
# There are 8 numbers corresponding to the 8 possible comparisons above
A = c(1, 0, 0, 0, 0, 0, 0, 0)
B = c(0, 0, 0, 0, 0, 0, 0, 1)

# contrast the marginal means
# infer argument returns a confidence interval and p value if
# both are set to TRUE.
contrast(marginal_means,
    method = list("Unadjusted effect size" = B-A),
    infer = c(TRUE, TRUE))
```

We could also make the more conservative assumption that any baseline trend continues by choosing the second row where baseline slope is set to the last treatment session.

```
# code to select first and last rows
# The 1 indicates that the row should be selected
A = c(0, 1, 0, 0, 0, 0, 0, 0)
B = c(0, 0, 0, 0, 0, 0, 0, 1)

# contrast the marginal means
# infer argument returns a confidence interval and p value if
# both are set to TRUE.
contrast(marginal_means,
    method = list("Unadjusted effect size" = B-A),
    infer = c(TRUE, TRUE))
```

Notice that there is much greater uncertainty in this contrast, evident by the increase in standard error, and as a result the p-value is no longer significant.

Group-level model

We can extend this individual model to all participants, still focusing on treated items in the blocked condition. First, we create a new data frame that includes all participants.

Then we can start again with a relatively maximal random effect structures, noting that we could also include random slopes for items. However, it is unlikely that such a model structure could be supported by the data. In this case we have chosen to include the most theoretically important random effects (Matsucheck, 2018) that we expect to be supported by the data. A formula for the model is:

$$\begin{split} \operatorname{response}_i &\sim \operatorname{Binomial}(n=1,\operatorname{prob}_{\operatorname{response}=1} = \widehat{P}) \\ \log \left[\frac{\hat{P}}{1-\hat{P}} \right] &= \alpha_{j[i],k[i]} + \beta_{1k[i]}(\operatorname{baseline_slope}) + \beta_{2k[i]}(\operatorname{level_change}_1) + \beta_{3k[i]}(\operatorname{slope_change}) \\ &\qquad \alpha_{j} \sim N\left(\mu_{\alpha_{j}},\sigma_{\alpha_{j}}^{2}\right), \text{ for item } \mathbf{j} = 1,\ldots, \mathbf{J} \\ \begin{pmatrix} \alpha_{k} \\ \beta_{1k} \\ \beta_{2k} \\ \beta_{3k} \end{pmatrix} &\sim N\begin{pmatrix} \begin{pmatrix} \mu_{\alpha_{k}} \\ \mu_{\beta_{1k}} \\ \mu_{\beta_{2k}} \\ \mu_{\beta_{3k}} \end{pmatrix}, \begin{pmatrix} \sigma_{\alpha_{k}}^{2} & \rho_{\alpha_{k}\beta_{1k}} & \rho_{\alpha_{k}\beta_{2k}} & \rho_{\alpha_{k}\beta_{3k}} \\ \rho_{\beta_{1k}\alpha_{k}} & \sigma_{\beta_{1k}}^{2} & \rho_{\beta_{1k}\beta_{3k}} & \rho_{\beta_{1k}\beta_{3k}} \\ \rho_{\beta_{2k}\alpha_{k}} & \rho_{\beta_{2k}\beta_{1k}} & \sigma_{\beta_{2k}}^{2} & \rho_{\beta_{2k}\beta_{3k}} \\ \rho_{\beta_{3k}\alpha_{k}} & \rho_{\beta_{3k}\beta_{1k}} & \rho_{\beta_{3k}\beta_{2k}} & \sigma_{\beta_{3k}}^{2} \end{pmatrix}, \text{ for participant } \mathbf{k} = 1, \ldots, \mathbf{K} \end{split}$$

The model takes a little longer to run, and returns a convergence warning

Again, we change the optimizer.

Since the model appears to have converged, we can examine the model results

summary(mod2)

```
## Generalized linear mixed model fit by maximum likelihood (Laplace
     Approximation) [glmerMod]
## Family: binomial (logit)
## Formula: response ~ baseline_slope + level_change + slope_change + (1 +
##
      baseline slope + level change + slope change | participant) +
##
       (1 | item)
##
     Data: df_glmm
## Control: glmerControl(optimizer = "bobyqa")
##
##
                      logLik deviance df.resid
        AIC
                BIC
##
     5652.2
             5755.8 -2811.1
                               5622.2
                                           7385
##
## Scaled residuals:
      Min
               10 Median
                                3Q
##
                                      Max
## -8.1144 -0.3551 -0.1630 0.3241 13.5510
##
## Random effects:
                              Variance Std.Dev. Corr
## Groups
               Name
##
   item
                (Intercept)
                              1.611099 1.26929
   participant (Intercept)
                              0.404805 0.63624
##
                baseline_slope 0.002648 0.05146
                                                0.41
##
                level change1 2.390503 1.54613
                                                 0.18 0.86
                              0.007304 0.08546 -0.78 -0.54 -0.52
##
                slope_change
## Number of obs: 7400, groups: item, 322; participant, 20
##
## Fixed effects:
                 Estimate Std. Error z value Pr(>|z|)
##
## (Intercept)
                 -3.44259
                              0.20649 -16.672 < 2e-16 ***
                                       4.050 5.13e-05 ***
## baseline_slope 0.07624
                              0.01883
                 0.85952
                              0.39754
                                        2.162 0.030611 *
## level_change1
## slope_change
                  0.09124
                              0.02582
                                       3.534 0.000409 ***
## Signif. codes: 0 '***' 0.001 '**' 0.05 '.' 0.1 ' ' 1
##
```

The summary table shows us there there are statistically significant effects for all three parameters: a small but significant trend at baseline, a fairly substantial level change on average, and an increase in slope from baseline that is slightly more than double the initial average trend. We also note that there there is much more variation in the level change parameter between participants relative to the baseline slope and slope change parameters. Finally, the correlation of fixed effects shows a positive association between individuals baseline trend and their level change, but a negative association between individuals baseline trend and slope change and level change.

We can calculate an overall effect size using the same approach as the individual model. In this case, we assume the median number of baseline sessions (11) and treatment sessions (20)

```
baseline_slope level_change slope_change emmean
##
                                                          SE df asymp.LCL asymp.UCL
##
                11 0
                                            0 -2.6040 0.268 Inf
                                                                    -3.1289
                                                                            -2.07905
                                                                    -2.2293
##
                31 0
                                            0 -1.0792 0.587 Inf
                                                                              0.07091
                11 1
##
                                            0 -1.7444 0.545 Inf
                                                                    -2.8119
                                                                             -0.67695
##
                31 1
                                            0 -0.2197 0.814 Inf
                                                                   -1.8145
                                                                              1.37515
##
                11 0
                                           20 -0.7791 0.393 Inf
                                                                   -1.5503
                                                                            -0.00791
##
                31 0
                                           20
                                               0.7457 0.410 Inf
                                                                    -0.0587
                                                                              1.54996
##
                11 1
                                           20
                                               0.0804 0.480 Inf
                                                                    -0.8613
                                                                              1.02214
##
                31 1
                                           20
                                              1.6052 0.581 Inf
                                                                    0.4669
                                                                              2.74347
##
## Results are given on the logit (not the response) scale.
## Confidence level used: 0.95
```

Because the baseline trend, on average, was statistically reliable, we calculated an overall effect size assuming that it would have continued in the absence of treatment.

```
# code to select first and last rows
# The 1 indicates that the row should be selected
A = c(0, 1, 0, 0, 0, 0, 0, 0)
B = c(0, 0, 0, 0, 0, 0, 0, 1)

# contrast the marginal means
# infer argument returns a confidence interval and p value if
# both are set to TRUE.
contrast(marginal_means,
    method = list("Unadjusted effect size" = B-A),
```

```
infer = c(TRUE, TRUE))
```

This results in a statistically reliable group effect size of 2.7 logits. Given that the group model suggests a starting place of only around 3% (found by converting logits to percentage plogis(-3.44)), this indicates a gain of about 35 percentage points on average can be attributed to the level and slope changes. We can estimate change in percentage points by adding the intercept, the baseline slope times an average number of baseline sessions, and the predicted change, converting to the predicted percent correct, and then subtracting the percent correct at baseline plus the baseline change: plogis(-3.44 + 0.07*5 + 2.68)-plogis(-3.44 + 0.07*5). However, we're not aware of a straightforward method of estimating individual effect sizes and confidence intervals using the frequentist approach.

Bayesian Mixed effects models

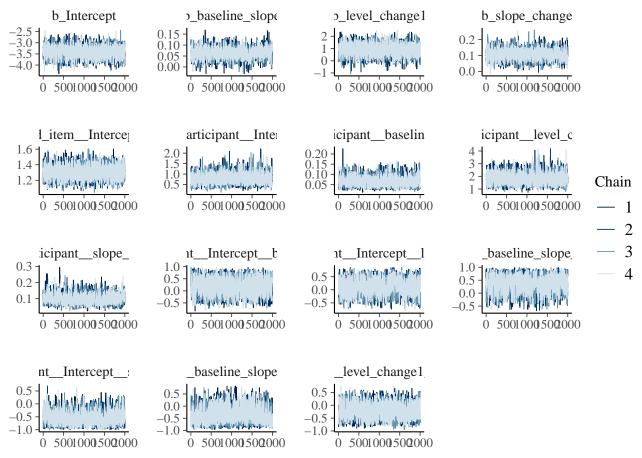
Bayesian mixed-effects models can be used in the same fashion as model 2 above to obtain both group and individual effect size estimates. First, a group-level model is estimated.

```
mod3 <-
brm(response ~ 0 + Intercept +
     baseline_slope + level_change + slope_change +
     (1 + baseline_slope + level_change + slope_change | participant) +
     (1 litem).
             data = df glmm,
             family = bernoulli(),
             iter = 3000,
             warmup = 1000,
             chains = 4,
             seed = 42.
             prior = c(
               prior(normal(-1, 2), class = b, coef = Intercept),
               prior(normal(0, 2.5), class = b)
             ),
             # extra arguments, see rmd file
             cores = 4,
             file = "models/group_brm",
             file_refit = "on_change")
```

We can check several aspects of model fit and convergence.

1. Traceplots: Note that there are four lines within each plot, representing all the samples from each Markov Chain Monte Carlo (MCMC) simulation from the above model. There should be no noticeable variations in the patterns of the estimates across each model. Visual inspection of convergence is achieved by affirming that the MCMC estimates were consistently sampled from a range of values. Graphically, this is presented by the horizontal structure in each trace plot. For example, all estimates of model 3s intercept, b_Intercept fall within a range of about -4 and -2.5 logits. "Spikes" observed in traceplots are normal, as can be seen in the participant_slope plot. These spikes represent estimates at the tail end of a probability distribution. Traceplots with positive or negative slopes or sinusoidal wave shapes do not reflect convergence.

```
brms::mcmc_plot(mod3, type = "trace")
```



2. Check that the model can successfully re-estimate the data. In this case, we conducted posterior predictive checks on three statistics that can be estimated from the model. The posterior_predict function creates n number of posterior distributions, where n is the total number of post warmup iterations for one MCMC chain. We label these distributions yrep in the code below.

Then, we estimate posterior predictive p-values for the mean, standard deviation, and kurtosis of the n number of posteriors included in yrep. We note, that this is only one type of method of posterior checking, and was chosen because graphical posterior predictive checks of the predicted number outcome estimates from logistic regression models involving a large number of 0s can be misleading.

```
y = mod3$data$response
yrep = posterior_predict(mod3)
mean_ppc = mean(apply(yrep, 1, mean) > mean(y))
sd_ppc = mean(apply(yrep, 1, sd) > sd(y))
kurtosis_ppc = mean(apply(yrep, 1, e1071::kurtosis) > e1071::kurtosis(y))
print(c(mean_ppc, sd_ppc, kurtosis_ppc))
```

[1] 0.506625 0.506625 0.482625

mean_ppc, sd_ppc, and kurtosis_ppc represent the proportion of mean, standard deviation, and kurtosis estimates less than the observed estimates of these parameters. The p-value is the proportion of posterior samples with greater prediction error than the actual data. Low p-values mean worse model fit than expected if the model were correct.

3. Rhat statistic should be < 1.05, ideally < 1.01

The rhat stastistic represents the potential scale reduction factor across the split chains, the case of this

model across the four chains. A value of 1.0 represents perfect convergence and estimates between 1 and 1.05, and ideally between 1 and 1.01 represent adequate convergence. Model fitting should be reevaluated with rhat statistics greater than 1.05.

```
max(rhat(mod3))
```

[1] 1.003422

We can preview the model results using summary() again. Notably, the model estimates are largely similar to the frequentist model.

summary(mod3)

##

```
##
    Family: bernoulli
##
     Links: mu = logit
## Formula: response ~ 0 + Intercept + baseline_slope + level_change + slope_change + (1 + baseline_slo
      Data: df glmm (Number of observations: 7400)
##
##
     Draws: 4 chains, each with iter = 3000; warmup = 1000; thin = 1;
##
            total post-warmup draws = 8000
##
## Group-Level Effects:
   ~item (Number of levels: 322)
                 Estimate Est.Error 1-95% CI u-95% CI Rhat Bulk_ESS Tail_ESS
##
                                                   1.46 1.00
##
  sd(Intercept)
                      1.30
                                0.08
                                          1.15
                                                                  2366
                                                                            4243
##
## ~participant (Number of levels: 20)
                                       Estimate Est.Error 1-95% CI u-95% CI Rhat
##
## sd(Intercept)
                                           0.77
                                                     0.23
                                                               0.40
                                                                        1.28 1.00
## sd(baseline_slope)
                                           0.06
                                                     0.02
                                                               0.03
                                                                        0.11 1.00
## sd(level_change1)
                                           1.80
                                                     0.40
                                                               1.12
                                                                        2.70 1.00
## sd(slope_change)
                                           0.10
                                                     0.03
                                                               0.05
                                                                        0.16 1.00
## cor(Intercept, baseline slope)
                                           0.21
                                                     0.33
                                                              -0.45
                                                                        0.79 1.00
## cor(Intercept,level_change1)
                                                     0.28
                                                              -0.43
                                                                        0.65 1.00
                                           0.15
## cor(baseline slope, level change1)
                                           0.36
                                                     0.32
                                                              -0.32
                                                                        0.87 1.00
## cor(Intercept,slope_change)
                                          -0.56
                                                     0.25
                                                              -0.93
                                                                        0.02 1.00
## cor(baseline_slope,slope_change)
                                          -0.37
                                                     0.31
                                                              -0.85
                                                                        0.33 1.00
## cor(level_change1,slope_change)
                                          -0.22
                                                     0.28
                                                              -0.72
                                                                        0.35 1.00
                                       Bulk ESS Tail ESS
## sd(Intercept)
                                                    3834
                                           2463
## sd(baseline_slope)
                                           2559
                                                    3731
## sd(level_change1)
                                           3090
                                                    4799
## sd(slope_change)
                                           1180
                                                    2898
## cor(Intercept,baseline_slope)
                                           1883
                                                    3701
## cor(Intercept,level_change1)
                                           1310
                                                    2675
## cor(baseline_slope,level_change1)
                                            915
                                                    1814
## cor(Intercept,slope_change)
                                           1053
                                                    2559
## cor(baseline_slope,slope_change)
                                            954
                                                    1958
## cor(level_change1,slope_change)
                                           1657
                                                    2921
##
## Population-Level Effects:
##
                  Estimate Est.Error 1-95% CI u-95% CI Rhat Bulk ESS Tail ESS
## Intercept
                                          -3.89
                      -3.41
                                 0.24
                                                   -2.96 1.00
                                                                   2118
                                                                            3857
## baseline_slope
                       0.06
                                 0.02
                                           0.02
                                                    0.11 1.00
                                                                   1309
                                                                            2171
## level_change1
                       0.90
                                 0.45
                                          -0.01
                                                    1.78 1.00
                                                                   2587
                                                                            3970
## slope change
                                           0.05
                                                    0.17 1.00
                                                                   1582
                       0.11
                                 0.03
                                                                            2628
```

```
## Draws were sampled using sampling(NUTS). For each parameter, Bulk_ESS
## and Tail_ESS are effective sample size measures, and Rhat is the potential
## scale reduction factor on split chains (at convergence, Rhat = 1).
```

Finally, we can calculate effect sizes from the model for each individual

To do this efficiently, we wrote a function which takes arguments for the model object, and an argument we called "adjust" that can be TRUE if we would like to extrapolate the baseline slope through the end of treatment and FALSE otherwise. The default is FALSE.

The function works be selecting rows for each participant and item in the data and estimating the posterior distribution for the values in each row.

Then the data is transformed and the posterior distribution at the beginning of treatment (or at the end of treatment without the level change and slope change parameters) is subtracted from the posterior distribution at the end of treatment.

The resulting posterior distribution characterized the magnitude of change, the mean or median can be used as a point estimate and the middle 95% of the distribution is the 95% credible interval.

```
getES = function(fit, adjust = FALSE){
  # start with the data that went into the model,
  # for each participant and phase (here we just used level_change
  # because they are equivalent) make a new variable called last_session
  # which is the highest value of the baseline slope coefficient
  # then filter for only rows where the baseline slope is
  # equal to the highest value in the phase (in other words
  # this selects the last baseline and last treatment session).
  # Remove the response column, reduce the data frame to only the
  # unique rows
  data = fit$data %>%
     group_by(level_change, participant) %>%
     mutate(last session = max(baseline slope)) %>%
     filter(baseline_slope == last_session) %>%
      select(-response) %>%
     distinct()
  # If adjust is TRUE, then for each participant,
  # set the baseline slope to always equal the highest value
  # of baseline slope.
  # in other words, we end up with a row where baseline slope
  # represents the last treatment session, but level and slope
  # change are still zero.
  if(adjust){
  data = data %>%
      group by(participant) %>%
     mutate(baseline slope = max(baseline slope))
  }
  # calculate an effect size in logits or log-odds
  # start with the data frame we just created and add
  # model draws from the linear/link-level predictor
  # change timepoint to entry if level change is
  # 0 and exit if 1. Select only the needed columns
  # and take the data from long to wide. using pivot_wider
  # Then subtract the value for the draw for entry from
  # the draw for exit.
```

```
# then for each participant, calculate the point
  # estimate and interval using point_interval
  # the last line just adds a column indicating this effect size is in logits
  linepred = data %>%
   add_linpred_draws(fit) %>%
   ungroup() %>%
   mutate(timepoint = ifelse(level_change == 0, "entry", "exit")) %>%
   select(timepoint, item, .draw, .linpred, participant) %>%
   pivot_wider(names_from = "timepoint", values_from = .linpred) %>%
   mutate(ES = exit-entry) %>%
   group_by(participant) %>%
   point_interval(ES) %>%
   mutate(unit = "logit")
  # This block does the same thing, except using the expectation of the
  # posterior (i.e., in percent correct terms)
  epred = data %>%
   add_epred_draws(fit) %>%
   ungroup() %>%
   mutate(timepoint = ifelse(level_change == 0, "entry", "exit")) %>%
   select(timepoint, .draw, item, .epred, participant) %>%
   pivot_wider(names_from = "timepoint", values_from = .epred) %>%
   mutate(ES = exit-entry) %>%
   group_by(participant) %>%
   point_interval(ES) %>%
   mutate(unit = "percent")
  return(bind_rows(linepred, epred))
Here's how we might use the function, adjusting for baseline slope
bayesian_es = getES(mod3, adjust = TRUE)
Examine the results for logits
head(bayesian_es %% filter(unit == "logit"), 20) %>% kable(format = "latex", booktabs = TRUE) %%
  kable_styling(position = "center")
Examine the results for percent
head(bayesian_es %>% filter(unit == "percent"), 20) %>% kable(format = "latex", booktabs = TRUE) %>%
  kable_styling(position = "center")
Save tables and plots for cleaning for publication
save(p1, p2, file = here("manuscript", "tables-plots.RData"))
sessionInfo()
## R version 4.2.0 (2022-04-22)
## Platform: x86 64-apple-darwin17.0 (64-bit)
## Running under: macOS Big Sur/Monterey 10.16
## Matrix products: default
           /Library/Frameworks/R.framework/Versions/4.2/Resources/lib/libRblas.0.dylib
## BLAS:
## LAPACK: /Library/Frameworks/R.framework/Versions/4.2/Resources/lib/libRlapack.dylib
```

participant	ES	.lower	.upper	.width	.point	.interval	unit
P1	2.1895495	-0.3676083	4.988229	0.95	median	qi	logit
P10	4.0086213	1.5187068	6.748027	0.95	median	qi	logit
P11	2.9714045	1.5204160	4.609804	0.95	median	qi	logit
P12	3.0598628	1.9864749	4.130898	0.95	median	qi	logit
P13	5.8589987	4.3140540	7.506057	0.95	median	qi	logit
P14	3.9626795	2.6624822	5.335825	0.95	median	qi	logit
P15	2.2477880	0.6808346	3.828825	0.95	median	qi	logit
P16	7.2926630	4.4957225	11.309594	0.95	median	qi	logit
P17	6.2582404	3.5445388	10.266857	0.95	median	qi	logit
P18	1.5851814	-0.0863041	3.492992	0.95	median	qi	logit
P19	5.4483097	2.8257044	8.691806	0.95	median	qi	logit
P2	5.7103733	2.8251632	9.211314	0.95	median	qi	logit
P20	3.4349465	2.2776165	4.745630	0.95	median	qi	logit
P3	5.0591621	2.4579499	8.537965	0.95	median	qi	logit
P4	4.6388253	2.4688143	7.444193	0.95	median	qi	logit
P5	2.8040056	1.1536990	4.869518	0.95	median	qi	logit
P6	3.7047279	1.2918042	6.876803	0.95	median	qi	logit
P7	0.2205605	-2.6562366	2.434720	0.95	median	qi	logit
P8	2.2802549	-0.1926789	4.786389	0.95	median	qi	logit
P9	1.1980474	-1.1147377	3.580816	0.95	median	qi	logit

participant	ES	.lower	.upper	.width	.point	.interval	unit
P1	0.3181775	-0.0483361	0.7443294	0.95	median	qi	percent
P10	0.4250740	0.0199313	0.8940472	0.95	median	qi	percent
P11	0.0869928	0.0082954	0.6206214	0.95	median	qi	percent
P12	0.5262244	0.1291868	0.7416138	0.95	median	qi	percent
P13	0.8543259	0.5419068	0.9424602	0.95	median	qi	percent
P14	0.6685233	0.2632803	0.8466362	0.95	median	qi	percent
P15	0.3431008	0.0433472	0.6799142	0.95	median	qi	percent
P16	0.8872033	0.5491569	0.9820621	0.95	median	qi	percent
P17	0.5681732	0.0907430	0.9129990	0.95	median	qi	percent
P18	0.0834094	-0.0056740	0.3588588	0.95	median	qi	percent
P19	0.7742871	0.1480239	0.9677798	0.95	median	qi	percent
P2	0.5197516	0.0352241	0.9583102	0.95	median	qi	percent
P20	0.4809946	0.0834361	0.7640616	0.95	median	qi	percent
P3	0.7847071	0.2275039	0.9540422	0.95	median	qi	percent
P4	0.7240730	0.1448200	0.9362476	0.95	median	qi	percent
P5	0.4817848	0.0932917	0.7780038	0.95	median	qi	percent
P6	0.6157490	0.2034815	0.8813546	0.95	median	qi	percent
P7	0.0327783	-0.2965442	0.5060892	0.95	median	qi	percent
P8	0.3184706	-0.0086866	0.7888893	0.95	median	qi	percent
P9	0.1379460	-0.1858118	0.6055480	0.95	median	qi	percent

```
##
## locale:
## [1] en_US.UTF-8/en_US.UTF-8/en_US.UTF-8/C/en_US.UTF-8/en_US.UTF-8
## attached base packages:
                 graphics grDevices utils
## [1] stats
                                                datasets methods
                                                                    base
## other attached packages:
   [1] forcats_0.5.1
                           stringr_1.4.0
                                               dplyr_1.0.9
                                                                  purrr_0.3.4
##
   [5] readr_2.1.2
                           tidyr_1.2.0
                                               tibble_3.1.7
                                                                  ggplot2_3.3.6
   [9] tidyverse_1.3.1
                           ggdist_3.1.1
                                               tidybayes_3.0.2
                                                                  brms_2.17.0
## [13] Rcpp_1.0.8.3
                           emmeans_1.7.3
                                               lme4_1.1-29
                                                                  Matrix_1.4-1
## [17] SingleCaseES_0.5.0 here_1.0.1
                                               kableExtra_1.3.4
##
## loaded via a namespace (and not attached):
##
     [1] readxl_1.4.0
                              backports_1.4.1
                                                    systemfonts_1.0.4
##
     [4] plyr_1.8.7
                                                    splines_4.2.0
                              igraph_1.3.1
##
     [7] svUnit 1.0.6
                              crosstalk 1.2.0
                                                    TH.data 1.1-1
##
   [10] rstantools_2.2.0
                              inline_0.3.19
                                                    digest_0.6.29
    [13] htmltools 0.5.2
                              fansi_1.0.3
                                                    magrittr_2.0.3
##
  [16] checkmate_2.1.0
                              tzdb_0.3.0
                                                    modelr_0.1.8
                              matrixStats_0.62.0
  [19] RcppParallel_5.1.5
                                                    vroom_1.5.7
##
  [22] xts_0.12.1
                              sandwich_3.0-1
                                                    svglite_2.1.0
##
   [25] prettyunits_1.1.1
                              colorspace_2.0-3
                                                    rvest 1.0.2
## [28] haven_2.5.0
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   [31] callr_3.7.0
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##
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##
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##
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                                                    proxy_0.4-26
##
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                                                    StanHeaders_2.21.0-7
##
   [52] DT_0.23
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                                                    httr_1.4.3
##
   [55] threejs_0.3.3
                                                    posterior_1.2.1
                              arrayhelpers_1.1-0
##
                                                    loo 2.5.1
    [58] ellipsis 0.3.2
                              pkgconfig_2.0.3
##
  [61] farver_2.1.0
                              dbplyr_2.1.1
                                                    utf8_1.2.2
## [64] labeling 0.4.2
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                                                    rlang 1.0.2
## [67] reshape2_1.4.4
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                                                    cellranger_1.1.0
##
   [70] munsell_0.5.0
                              tools_4.2.0
                                                    cli_3.3.0
## [73] generics_0.1.2
                              broom_0.8.0
                                                    ggridges_0.5.3
## [76] evaluate 0.15
                              fastmap_1.1.0
                                                    yaml_2.3.5
## [79] bit64 4.0.5
                                                    processx 3.5.3
                              fs 1.5.2
## [82] knitr 1.39
                              nlme_3.1-157
                                                    mime 0.12
## [85] projpred_2.1.1
                              xml2_1.3.3
                                                    compiler_4.2.0
## [88] bayesplot_1.9.0
                              shinythemes_1.2.0
                                                    rstudioapi_0.13
                              e1071_1.7-9
##
   [91] gamm4_0.2-6
                                                    reprex_2.0.1
                              ps_1.7.0
                                                    Brobdingnag_1.2-7
##
   [94] stringi_1.7.6
  [97] lattice_0.20-45
                              nloptr_2.0.0
                                                    markdown_1.1
## [100] shinyjs_2.1.0
                              tensorA_0.36.2
                                                    vctrs_0.4.1
## [103] pillar_1.7.0
                              lifecycle_1.0.1
                                                    bridgesampling_1.1-2
## [106] estimability_1.3
                                                    R6_2.5.1
                              httpuv_1.6.5
## [109] promises_1.2.0.1
                              gridExtra 2.3
                                                    codetools 0.2-18
## [112] boot_1.3-28
                              colourpicker_1.1.1
                                                    MASS_7.3-56
## [115] gtools 3.9.2
                              assertthat_0.2.1
                                                    rprojroot_2.0.3
```

##	[118]	withr_2.5.0	shinystan_2.6.0	multcomp_1.4-19
##	[121]	hms_1.1.1	mgcv_1.8-40	parallel_4.2.0
##	[124]	grid_4.2.0	class_7.3-20	coda_0.19-4
##	[127]	minqa_1.2.4	rmarkdown_2.14	<pre>lubridate_1.8.0</pre>
##	[130]	shiny_1.7.1	base64enc_0.1-3	dygraphs_1.1.1.6